Turning the Tide: Harnessing the power of child health research

A report by the Royal College of Paediatrics & Child Health Commission on Child Health Research
“Turning the Tide”:
Harnessing the power of child health research

A report from the RCPCH Commission on Child Health Research
## Abbreviations

<table>
<thead>
<tr>
<th>Abbreviation</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>MCRN</td>
<td>Medicines for Children Research Network</td>
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<td>MHRA</td>
<td>Medicines and Healthcare products Regulatory Agency</td>
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<td>NICE</td>
<td>National Institute for Clinical Excellence</td>
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<td>NHS</td>
<td>National Health Service</td>
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<td>NIHRN</td>
<td>National Institute for Health Research</td>
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<td>OOPR</td>
<td>Out-of-Programme-for-Research</td>
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<td>OOPE</td>
<td>Out-of-Programme-for-Education</td>
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<td>RCPCH</td>
<td>Royal College of Paediatrics and Child Health</td>
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<td>UKCRC</td>
<td>UK Clinical Research Collaboration</td>
</tr>
</tbody>
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Foreword

The title of this report is apt - ‘harnessing the power of child health research’. The power of researchers is to identify uncertainties, ask important questions, measure health and disease, and responses to prevention and treatment. From vaccination to the treatment of childhood leukaemia, research in children has saved and improved countless lives.

Education and training in research is also integral to the everyday practice of the best medicine. A key characteristic of an excellent practitioner is that, by knowing that much is not known, medicine is practised with an enquiring mind and a non-dogmatic approach. These are key manifestations of professionalism. The best care depends on applying the outcomes of the best research - that is how research achieves its impact.

But in order to harness the power of research, it is essential that there is research to harness in the first place. Here there is something of a crisis, in that the fraction of paediatricians who are training in research is too small, and the community of leaders to inspire and mentor new generations of researchers is ageing and shrinking.

A strength of this report is that it looks forward and is not excessively introspective about the past. Rightly the first set of recommendations start ‘at home’ with a set of commitments from the College itself. One of the most important barriers to career development in clinical research is the inflexibility of the clinical training pathway and, of course, this is not a problem limited to paediatric training.

Those of us currently in senior academic roles were trained in the days before logbooks and rigid training rotations. We recognised that training was a lifelong affair and that as newly appointed consultants, we were not perfectly formed and did not have ubiquitous clinical skills. This report is timely because the RCPCH has the opportunity to work with the GMC, and particularly with the current review of postgraduate medical training and education review led by Professor David Greenaway to develop a much more flexible approach to the training of the paediatricians of the future. This will be essential if we are to produce the next generation of paediatric academic leaders, skilled in research and excellent clinical practitioners.

The report notes that the fraction of research funding devoted to paediatrics and child health by the major research funders with a broad health research mission is relatively small, and that includes the Wellcome Trust. But we do not ‘ring fence’ our funds for particular clinical areas. We are driven by excellence. However, it is extremely encouraging that paediatrics and child care does well in the competition for academic clinical fellows, because many of these will be the leaders of the future. These outstanding trainees are showing that they can compete successfully for funds from the MRC, NIHR, Wellcome Trust, CRUK and other funders. It is important that the RCPCH is taking such a close interest in these and other academic trainees, and this must be sustained.

Some of the problems identified in this report are specific to paediatrics and child health, others are generic. One key problem is that, whilst we continue to label time spent in research training as ‘out of programme’, those responsible for supervising training will continue to be ‘doing a favour’ to trainees by allowing time for research training.

What is really needed is strong leadership with a long-time horizon to solve these important problems. This report signals to me that the RCPCH intends to play a major leadership role. I applaud this. The College must foster and support academic trainees.
Research training must move back from the exception to the norm. But I finish with some challenges. Why is postgraduate clinical training in the UK so much longer than in many other countries? As this report shows, it is not that child health is better in the UK than elsewhere. Can we stop labelling research training as ‘out of programme’ and limiting the access of trainees to time devoted to research? I look forward to seeing an increased role of the RCPCH in ensuring that the UK really does harness the power of research.

Mark Walport, Director of the Wellcome Trust, November 2012
## Contents

<table>
<thead>
<tr>
<th>Section</th>
<th>Page</th>
</tr>
</thead>
<tbody>
<tr>
<td>Executive summary</td>
<td>1</td>
</tr>
<tr>
<td>1  Child health research</td>
<td>2</td>
</tr>
<tr>
<td>2  Biomedical research in the UK</td>
<td>3</td>
</tr>
<tr>
<td>3  Children's research activity</td>
<td>4</td>
</tr>
<tr>
<td>4  Research training</td>
<td>5</td>
</tr>
<tr>
<td>5  Research and the NHS</td>
<td>6</td>
</tr>
<tr>
<td>6  University employed paediatricians</td>
<td>7</td>
</tr>
<tr>
<td>7  Young people, parents and the public</td>
<td>8</td>
</tr>
<tr>
<td>8  Translating research into policy and clinical practice</td>
<td>9</td>
</tr>
<tr>
<td>9  Increasing and strengthening child health research: the way forward</td>
<td></td>
</tr>
<tr>
<td>10 References</td>
<td>10</td>
</tr>
<tr>
<td>11 Contributors</td>
<td>11</td>
</tr>
<tr>
<td>12 Acknowledgements</td>
<td>12</td>
</tr>
</tbody>
</table>
Child health research

Introduction

Children deserve, and parents expect, the best healthcare for their children. Over the last 50 years there has been a substantial increase in our understanding of illness and our ability to treat or modify many diseases successfully. Our care of the new-born has also improved with many more neonates surviving now than previously. Much of this progress has been underpinned by research led by paediatricians but, in spite of children never having been so healthy, there is much more that can and should be done. Current health indicators suggest that we have fallen from our position as a leader in child health to one where our outcomes are poorer than most of our European neighbours. We need research to tell us why. There has also been a revolution in our scientific understanding and ability to investigate disease, and in the way that research in the NHS has been funded, but there are worrying signs that paediatricians have taken insufficient advantage of these opportunities. The RCPCH recognises that its primary role is the education and training of tomorrow’s paediatricians, and advocacy for the best healthcare for babies and children. Research is vital to progress and the RCPCH has therefore produced this report, identifying the issues, and making recommendations as to how we might take advantage of the opportunities available.

Child health research

Child health defines wellbeing across the life-course. In the UK, children are not well served, with higher all-cause mortality in comparison to other European countries, and the lowest ranking position for all measures of child wellbeing among 21 countries in the industrialized world. Children are not small adults; they need biomedical and health services research that takes account of their changing physiology, and addresses their problems directly, generating evidence to improve the quality of the treatments and healthcare they receive, and the policies that affect their wellbeing. There are other reasons why children’s research is important to the nation. Early life exposures, many mediated through disadvantage and deprivation, impact on adult health and on succeeding generations. The growing burden of chronic, long-term conditions that have a substantial component of their origins in early life, obesity, cardiovascular disease, vascular dementia, and diabetes, are placing an intolerable strain upon the National Health Service (NHS) and adversely affecting the health and economic wellbeing of the nation.

Recent advances

Children's research is needed to define the causal biological mechanisms, alter the development of aberrant trajectories, preserve health, and reduce the costs of healthcare in adult life. The UK population is aging; health and wellbeing in old age are a matter of growing national concern. Sciences that hold great promise for health in old age, such as tissue regeneration, stem cell biology, neural plasticity, and immune modulation, require basic research that begins in infancy.

In recent years there has been an explosion in powerful technologies, in-vivo imaging, non-invasive monitoring, high-throughput analytical techniques employing tiny sample volumes, bio-informatics, and epigenetics, that provide opportunity to involve children in research as never before, and unravel the molecular basis of links between early life exposures, development, deprivation, and disease. In parallel, the organisational structures of the NHS, the largest universal healthcare system in the world, provide a unique platform to integrate clinical research and patient care, speed the translation of new treatments into practice, and test preventive interventions rigorously. Research harnessing the wealth of post-genomic sciences and the power of the NHS, offers unparalleled opportunity to improve the wellbeing of infants and children, turn the tide of the growing burden of the
major non-communicable chronic diseases that have their origins in early life and lead to premature adult death, and benefit the health of future generations.

The Royal College of Paediatrics and Child Health Commission on Child Health Research was established in view of concerns about child health biomedical and health services research in the UK, and charged with considering how this might be strengthened and increased. We evaluated training, infrastructure and capacity, support within the NHS, the extent to which paediatricians are able to support clinical research, activity and funding, parent, public and young people's involvement, whether national clinical guidelines and policies affecting child health are adequately informed by research evidence, and the visibility of children's research.

The UK landscape

Education and training

We found superb opportunities for the research leaders of the future through the ‘Integrated Academic Training Pathway’, excellent support for the delivery of medicines studies through the National Institute for Health Research Medicines for Children Research Network, sterling examples of NHS consultant paediatricians supported to become research leaders, trainee paediatricians eager to be involved in research, major contributions to the international scientific literature by UK paediatricians, multiple research funding streams available through the National Institute for Health Research and research councils, dedicated charities, parents and young people keen to be active partners, and a strong Government commitment to biomedical research. All of this should be applauded loudly.

We also identified problems. There is frustration in a rigid postgraduate medical training system that offers poor opportunity for experience and education in core skills to support clinical research. Only a small minority of paediatric clinical trainees report having received teaching in basic research methods, research regulation, organisation and governance, and in their final years of training many are not confident in basic skills such as taking informed consent for research participation. In previous years all consultants were expected to be involved in research; today the majority of newly appointed consultant paediatricians have little or no research experience and only one in ten has a higher research degree, compared with one in three paediatricians nearing retirement.

Capacity and infrastructure

There has been a decline in children's research capacity in the UK with few university posts to realise the potential of the next generation of world-class researchers. Since 2000, there has been an 18% reduction in the number of university-employed child health researchers and a fall in the proportion of university employed paediatricians from 8.7% of the total consultant-level workforce in 2001 to 5.2% in 2011. There are now only about two hundred paediatric professors, readers, and senior lecturers in the UK and the number of lecturers, the research leaders of the future, has fallen to 28, the lowest level ever. Two-thirds of senior lecturers in paediatrics are above the age of 45 and over the next ten years 50% of current professors of paediatrics will retire, indicating the likelihood of further decline in leadership in children's research. Many child health research groups, while doing sterling work to promote research, are too small and lack critical mass. At the same time researchers report poor support for the development of collaborations, citing impediments arising from institutional rivalries and often insurmountable bureaucracy.

The infrastructure and support for children's basic science and non-medicines applied research is not consistent. There is, for example, little activity in children's primary care,
health technologies, and health services research. In the most recent round of awards in 2012, the National Institute of Health Research funded one children's Biomedical Research Centre and no children's Research Units. Regardless of whether this reflects the number and quality of the applications or strategic priorities, it is a situation that we need to improve upon in the future. Less than 5% of National Institute of Health Research portfolio studies and around one in ten applications for national research ethics approval involve children; in comparison, internationally, children's research represents 10-15% of all registered trial activity. Of the 20 children's hospitals in the UK, 10 have no children's research facility.

**Activity**

The complex organisational structure of the NHS means that despite strong leadership in attempting to improve both research integration and the regulatory framework, there is still marked variability in progress around the country, particularly in relation to the needs of infants, children, and young people. Support for the delivery of children's non-medicines clinical studies in the NHS through the National Institute for Health Research networks is also variable. It is extremely encouraging that around 50,000 children were recruited to research studies over 2011/12, a huge increase over previous years; however, this still represents less than 2.5% of the total 2 million NHS consultant episodes for children each year. Two-thirds of paediatric consultants have no time allocated to support clinical research, and overall less than 5% of all contracted consultant time is for this purpose. Many processes common to NHS care and children's research, such as data collection and follow-up assessments, are duplicated and this is an unnecessary and off-putting burden on families. The EU Regulation on Medicines for Paediatric Use (2007) was an important milestone in ensuring a Paediatric Investigation Plan for all new medicines, but still over 90% of medicines for infants are used off-label or off-license because the necessary clinical studies have not been carried out. A large number of clinical treatments in wide use lack an adequate evidence base. Many other low risk but essential studies, such as dosing data for widely used medicines, and long-term safety monitoring, are not being carried out because of a prohibitive regulatory environment, and escalating research costs consequent upon a growing bureaucracy, in which the benefit to patient care from a proportionate, less rigid, approach to risk assessment is inadequately recognised. Only a fifth of people are aware that research is a key activity for the NHS, though four-fifths consider it important to be offered the opportunity to participate. Two-thirds of healthcare professionals say that research is peripheral in their NHS Trust.

**Funding**

Funding for child health research is fragile, representing 5% of the annual UK public and charitable research expenditure of approximately £2.2 billion, equivalent to less than £10 per child each year. Commercial studies now make up 60% of the Medicines for Children Research Network portfolio, but we found no evidence of significant industry support for children's biotechnologies, devices, and nutrition research; there are no commercial studies in the National Institute for Health Research Paediatric Non-Medicines Portfolio. Paediatricians are twice as likely to receive a research grant from a local or national charity, than from the National Institute for Health Research, Medical Research Council, and Wellcome Trust combined, but of the large number of national children's research charities, only one has a research spend that exceeds £1.5 million per annum, largely precluding their ability to support large clinical trials and major research programmes, establish substantive research posts, create regular opportunities for research experience, or fund infrastructure.

Representation by paediatricians on major research boards is weak, and parent and young people's advocacy is fragmented. The scant evidence-base for child healthcare is impeding the development of effective national guidelines and policy with less than 20% of outputs from the National Institute of Clinical Excellence applicable to children. There is inadequate primary research evidence to support many clinical guidelines, inadequate translation of
scientific research into policy, and little in the way of objective assessment of the impact of policy on children's health outcomes.

The relevance of early years' research to children's immediate health, their health in adult life, the health of future generations, and the economic wellbeing of the nation is one of the great but hidden challenges in public life. We call on all those parties involved in research to come together to develop a national vision which will ensure this is recognised and acted upon.

Solutions

A call to action to all key stakeholders

The purpose of this report is not to apportion blame, not least when such progress has been made by all those working in the field. Our overarching aim is to catalyse action by bringing a spotlight to bear upon the importance to the nation of research to improve children's health and wellbeing. This matters not only to today's children, but equally to tomorrow's adults. Children's research is of critical importance if we are to have any prospect of turning the rising tide of chronic non-communicable diseases that have their determinants in early life, that lead to premature adult death and disability, and that place an increasing burden upon the NHS.

Only co-ordinated joint action will achieve this. We therefore call upon Royal Colleges, universities, the biotechnology, pharmaceutical and infant nutrition industries, philanthropists, research charities and research councils, the NHS and UK government, as well as parents, young people and the public to join us in creating a national vision which increases the evidence-base for health care and policy that affects the well-being of infants, children, and young people, their life-long health, and the health of future generations

We have identified six priorities that we must address collectively if the power of children's research to improve their wellbeing, and the health of the nation, is to be harnessed. In taking responsibility for these actions, it is right that we start with what we, the RCPCH will do to play our part.

The Royal College of Paediatrics and Child Health commitment

We recognise that many factors may have contributed to a fall in research capacity and capability, including some of the ethical and practical considerations surrounding research involving children, the historical and contemporary priorities of grant-giving bodies, and even some aspects of the culture of paediatrics. Therefore unless we start with our own commitment to supporting the development of the next generation of paediatric academics and to ensuring high calibre funding applications, no amount of broader national support will address the problems.
1. Education, training and guidance

We will improve training in research skills for all paediatricians in our training programmes. A Guide to Training in Child Health Research has been prepared and is widely available; an Academic Training Committee has been established to supervise activities and ensure these objectives are met. Specifically we will:

- ground paediatric training in the tools of science
- foster opportunities to join a clinical or non-clinical child health research group at undergraduate level, and during paediatric clinical training
- sign-post medical students and paediatric trainees to child health researchers who are able to provide research opportunity and supervision
- provide clear, consistent guidance on routes into research, and requirements for obtaining approval to take time out of clinical training to obtain research experience
- assess progress in attaining the core, generic research competencies included in the General Medical Council approved paediatric curriculum as part of the Annual Review of Competency Progression required of all paediatric trainees

We are updating our ‘Guidance for the Ethical Conduct of Research Involving Children’, an influential document first published in 1980, and revised in 2000. This will include a ‘Code of conduct for paediatricians working with industry’ to guide children’s researchers working with the infant nutrition, pharmaceutical, and medical devices industries.

2. Children and families

Children, young people and parents should be at the centre-stage of efforts to increase and strengthen research to benefit their life-long health. They are partners in the process and have a vital role to play in advocating for research to reduce uncertainties in their treatments and delivery of care. We will heed the messages from parents, young people and children about the way in which they wish to be involved in and be told about research, promote their engagement, and support them in conveying these messages to the research community and research regulators.

We applaud the recommendation for a Children’s Charter proposed by the Children’s Outcomes Forum, and will collaborate with key partners to ensure it reflects the importance of research. In particular we will work with our Youth Advisory Panel, our Patient and Carer’s Advisory Group and other relevant lay groups to ensure the Charter sets out the ways in which children, young people and their families can support research in order to better understand the biology of their health and disease. Just as importantly, the Charter must stress the critical importance of ensuring that research evidence is incorporated in a timely way into national clinical guidelines and policies, and that these are implemented, audited, and evaluated.
We have a duty as a College to support professionals in communicating with parents and children about the benefits of participating in research and will provide relevant guidance, training and support to ensure that all clinicians involved in research activity are confident in such communications.

3. Bringing organisations together

Children's research will benefit from a coordinated approach to tackling priorities, overcoming obstacles, identifying funding in challenging economic circumstances, and improving the evidence base for national policies that affect children. The Royal College of Paediatrics and Child Health is committed to fostering an ethos strongly supportive of research, widening the involvement of NHS clinicians, monitoring trends, assessing impact, and strengthening child health policy development. A new 'Science Advisory Board' will be established to direct these activities. A collaborative, strategic approach will help organisations work together to break obstacles to progress, maximise impact, sharpen the focus on children's research, and maintain momentum.

We have therefore begun consultations with children's research charities, and other organisations, on the establishment of a UK ‘Children's Research Collaboration’. We will support strategic alignment of smaller charities and large funding bodies to optimise returns from research funding for best long-term benefit, advocate and lobby, and raise awareness of the need to increase and strengthen children's research in the UK.

Our proposals to national bodies and the broader NHS

4. Infrastructure

The infrastructure for children's basic science and applied research in the UK is fragmented and complex. The Report of the Children's and Young People's Health Outcomes Forum highlighted the need to stimulate the development of academic child health, both physical and mental, and the evidence base for practice and improving outcomes. Alongside our own commitments to improving research capability, we believe that infrastructure needs to be improved. We recommend that national bodies consider the following options as a means of achieving this:

- the National Institute for Health Research, research councils, and universities support the formation of multidisciplinary, cross-institutional groupings of clinical and non-clinical child health researchers and their access to diagnostic and laboratory facilities suitable for children; an example of such an approach, which brings paediatricians and adult medicine researchers together, is the Southampton Life Course Respiratory Biomedical Research Unit, and the Nutrition, Diet and Lifestyle Biomedical Research Centre

- the National Institute for Health Research establishes a unified Children's Research Network to support the delivery of medicines and non-medicines children's studies
• the Health Research Authority ensures that regulation is mindful of the needs of infants, children and young people, risk assessment is proportionate, and review is based upon criteria that are consistent nationally

5. Capacity

Children’s research capacity is critically small with few posts for young researchers, and few substantive research grants awarded to paediatricians. We recommend:

• a collaborative effort by the National Institute of Health Research, universities, research councils and charities to bring about an acute expansion in clinical and non-clinical post-doctoral positions, lectureships and senior lectureships in child health, linked to established research groups to empower child health researchers to achieve success in a competitive and financially difficult environment

6. The National Health Service

The NHS should be the best place in the world for children’s research. Within the existing government strategy to bring about closer integration of children’s research with core NHS activities, we would highlight the following proposals and strategies as a means of achieving this end:

• The duplication of processes common to clinical care and research that add unnecessarily to NHS costs and are burdensome to families and healthcare staff, should be eliminated

• Clinical Reference Groups, the National Commissioning Board, Clinical Commissioning Groups, and Public Health England should specifically address integration of children’s research into clinical care within commissioning frameworks; this should include the opportunity for children to participate in studies to evaluate treatments that are already in wide use, obtain pharmacokinetic data on existing medicines, and conduct long-term safety monitoring. Disease surveillance to monitor morbidity and mortality rates for key diseases, should be considered a standard of care

• As part of this commitment, NHS commissioners are in a unique position to establish a national network of infant and children’s follow-up centres staffed by personnel trained to deliver neurodevelopmental and other assessments to a standard required for both clinical care and research outcome evaluation, and to ensure this information can be shared appropriately

• NHS providers should ensure children’s hospitals and departments have in-patient and out-patient research facilities suitable for infants, children, young people and families, as part of the Trust Board level commitment to research.

• NHS employers should adopt a flexible and individual approach to increase the contribution of paediatric consultants to children’s research whether as users, contributors, or leaders, and support them to access Continuing Professional
Development to maintain up-to-date knowledge of research regulation, organisation, and opportunities for trainees

- the National Institute for Health Research should recognise and reward high performing NHS Trusts for the capture of high quality clinical data used for both NHS purposes and research, in a manner analogous to the incentives received for recruitment to NIHR portfolio studies

We strongly believe that if we can achieve the national partnership working we have outlined, we can make the UK a world leader in children’s research that will be of incalculable benefit to children, their life-long health, and the health of the nation and of successive generations.

Professor Neena Modi, Vice-President (Science and Research), RCPCH

Dr Helen Budge, Professor Howard Clark, Dr Ingrid Wolfe, Professor Anthony Costello, on behalf of the RCPCH Commission on Child Health Research

November 2012
1 Child health research

“The moral case for investing in children is compelling” Save the Children 2012

1.1 There are many types of child health research (table 1.1). We use the term to include primary research addressing disease prevention and treatment, health services research addressing the organisation and delivery of healthcare, and health policy research that usually involves the synthesis of primary research evidence (secondary research).

Clinical research, that is research involving patients or healthy volunteers, may take the form of a clinical trial where different treatments, medicines or other interventions, are compared to establish which results in better outcomes. It may also be observational, where there is no intervention and the patient or healthy volunteer is monitored carefully with perhaps extra measurements or tests, in order to better understand the natural history of health and disease. Clinical pharmacokinetic research is necessary to determine the best way to administer medicines and the optimum dosing regimen. Qualitative research employs subjective measures such as when evaluating patient experiences.

Table 1.1 Types of child health research

<table>
<thead>
<tr>
<th>Description</th>
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<tr>
<td>Descriptive studies (epidemiology of disease, health indicator monitoring)</td>
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<tr>
<td>Elucidation of aetiology and mechanisms of disease (pre-clinical and clinical research)</td>
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<td>Development of interventions (development of medications, health technologies, preventive strategies)</td>
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<td>Efficacy studies (clinical trials)</td>
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<tr>
<td>Effectiveness research (phase III clinical trials, health services research, quality improvement programmes and case reviews, policy evaluations)</td>
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<tr>
<td>Qualitative research</td>
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<td>Policy research (primary and evidence synthesis)</td>
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</tbody>
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1.2 Child health research has historically taken a rear seat, with therapies initially developed for adults subsequently being adopted for children. In large part this arose from a desire to protect children from the dangers of experimental medicine, instances of unethical research in children, and the invasive nature of investigative techniques. This legacy no longer serves children well.

1.3 Technological advances such as high throughput analytical techniques employing very small volumes of biological fluids, and in-vivo imaging techniques, provide ever greater opportunities for non-invasive investigations ideally suited to infant and child research. When coupled with large electronic databases, bioinformatics techniques, and the identification of reliable biomarkers of outcome, infants and children can be included today in biomedical and clinical research to an extent previously impossible.

1.4 Children’s research is necessary because the biology of any given disease is not necessarily the same as in adults. Children’s physiology also alters with age, so that
for example, the doses of medicines and their actions are often quite different from adults. This can lead to tragic consequences when treatments designed for adults are delivered to children without adequate testing; one of the earliest antibiotics developed, chloramphenicol, while being extremely effective at treating infections in all age groups, is poorly metabolised in babies, resulting in serious illness and even death from the side effects of the medicine. Aspirin, widely used for pain relief and to reduce fever in adults, is not recommended for use in children because of its association with a serious condition, Reyes syndrome. Adolescents with cancer have significantly better survival when treated with protocols developed for children compared with protocols used for adults (Stock et al 2008).

1.4 New emphasis on the relevance of child health to population health has been brought about by the explosion in research over the last two decades that demonstrates the impact of infant and child health on adult wellbeing. Healthy infants are more likely to be healthy children and healthy adults; for example, 80% of obese children will become obese adults, and the children of obese women are more likely to become obese. The major causes of death and poor health in adult life, obesity, cardiovascular disease, and stroke, have their determinants in early development. The imperative to reduce the exponential increase in these lethal non-communicable diseases and improve population health is ample justification for increased research in infancy and childhood. There are wider implications of children’s research and understanding children’s diseases. Rehabilitative treatments that aim to exploit stem cell therapy, gene therapy, and tissue engineering require better understanding of developmental biology, neural plasticity, senescence, and tissue regeneration, sciences that are centred upon child research.

1.5 The view of previous years, that children should be protected from research, has been replaced with an understanding of the benefits to children that result from participation in methodologically rigorous, appropriately regulated medical research. The RCPCH has been among the foremost champions of this latter view, noting in 1980, at a time when the legality of research involving children was still being debated (Dworkin 1978; Skegg 1977) that “research involving children is important for the benefit of all children and should be supported and encouraged, and conducted in an ethical manner” and “research which involves a child and is of no benefit to that child (non-therapeutic research), is not necessarily either unethical or illegal” (Cockburn et al 1980).

1.7 A consideration that disproportionately affects children's research is that investigations aimed at understanding the natural history of disease, the efficacy and effectiveness of preventive health measures, and long-term outcomes of interventions and treatments, take many years. This adds to the costs of children's studies, making them unattractive to industry and smaller charities that require near immediate impact to satisfy business imperatives or to raise funds. For example, preterm chronic lung disease trials showed short-term improvement from steroid medication. However failure to fund follow-up assessments delayed the recognition that steroids also resulted to an almost three-fold increase in cerebral palsy (Barrington 2001).

1.8 From a national perspective the economic arguments in favour of children's research activity are compelling. Improving child health accumulates advantages throughout the life course and investment in the earliest years will reap the biggest rewards.
Summary

Infants, children and young people need treatments that are developed for them, interventions that are tested in them, health services that are designed for them and policies that address their health and wellbeing.

Many diseases and much ill-health in adult life have their origins in early development. Children's research offers unparalleled opportunity to improve life-long health and turn the tide of an increasing burden of non-communicable chronic diseases, obesity, cardiovascular disease, and diabetes, which lead to premature adult death.

Recommendations

Early year's research requires a national vision, which increases the evidence-base for health care and policy that affects the well-being of infants, children, and young people, their life-long health, and the health of future generations

- The participation of infants, children and young people in well designed, appropriately regulated research should be promoted to improve their health and wellbeing, health in adult life, and the health of successive generations
2 Biomedical research in the UK

“Improving the health and wealth of the nation through research” National Institute of Health Research Mission Statement

2.1 The UK has a long and successful history of clinical research though there have also been times of difficulty. The Academy of Medical Sciences raised concerns about the future of clinical research in the UK bringing the issue to the attention of the House of Lords Science and Technology Select Committee in 2001. Conducting clinical research in the UK had become much harder with a regulatory environment that was deterring investment in research despite the excellent potential of the NHS. The subsequent Academy of Medical Sciences report “Strengthening Clinical Research” (2003) was instrumental in leading to the establishment of the UK Clinical Research Collaboration in 2004 and the UK strategy “Best Research for Best Health” (2006). The aims were to re-engineer and place the NHS at the heart of the clinical research environment in the UK to benefit the public and patients.

2.2 The National Institute of Health Research (NIHR) was established with four work strands covering research training, support for researchers, funding for research projects and programmes, providing facilities and creating streamlined systems.

2.3 The NIHR Medicines for Children Research Network (MCRN) was created in 2005 as part of the drive to improve the environment for clinical research in the UK especially for the pharmaceutical industry. It is funded by the Department of Health and its primary remit is to “improve the UK’s clinical research environment and maximise the development of safe and effective medicines and formulations for children”

In Scotland research support is available through ScotCRN, the Scottish Medicines for Children Network and the Chief Scientist Office; in Northern Ireland through the Clinical Research Network (Children) and in Wales through the Welsh Children & Young People’s Research Network and National Institute for Social Care & Health Research Clinical Research Centre.

Medicines for Children Research Network
Dr Willian Van’t Hoff, Co-Director of the MCRN

Half of children’s medicines and approximately 90% of medicines for newborn babies are prescribed without licence or off-label. These inequalities were addressed in the EU Regulation on Medicines for Paediatric Use, which came into force in January 2007, requiring pharmaceutical companies to agree, with the European Medicines Agency, a Paediatric Investigation Plan for all new medicines at a very early stage in the development process. A marketing authorisation is only granted if a Paediatric Investigation Plan had been approved and the studies completed. In 2004, while this Regulation was going through the legislative process, the Medicines and Healthcare products Regulatory Agency (MHRA)/Department of Health Paediatric Strategy identified the need for a dedicated research network to support paediatric studies which would result from the implementation of the regulation. The MCRN was established as one of the four new topic-specific research networks within the NIHR Clinical Research Network. Approximately 90% of commercial studies supported by the MCRN are linked to an approved Paediatric Investigation Plan. The overall
The purpose of the MCRN is to facilitate the conduct of randomised prospective trials and other well-designed studies of medicines for children, including those for prevention, diagnosis and treatment, in order to enhance patient care by improving the quality, speed, coordination and integration of research. The partnership in place between the MCRN, six Local Research Networks and the Comprehensive Local Research Networks ensures that MCRN studies are supported across the whole of England.

2.4 The Paediatrics (Non-Medicines) Specialty Group is one of twenty-four Specialty Groups providing national networks of topic-specific expertise. The remit of the Paediatrics (Non-Medicines) Specialty Group is to support children’s research (excluding medicines studies) (http://www.crncc.nihr.ac.uk/about_us/ccrn/specialty/paediatrics/paediatrics.htm) but receives no direct funding. Paediatrics (Non-Medicines) studies may receive support through either the MCRN or the local CRN with decisions made on a case-by-case basis with resulting variable support reported by investigators.

2.5 The NIHR has funded Biomedical Research Centres based within NHS and University partnerships to lead in translating biomedical research into clinical research that benefits patients. Funding for the first round of Biomedical Research Centres extended from 2007-2012. Following a second competitive round, eleven Biomedical Research Centres receive current funding. Of these only one (University College London Institute of Child Health/Great Ormond Street) is a children’s Biomedical Research Centre, and among the others, Neonatal Medicine and Paediatrics are named as explicit themes only within the Imperial College London Biomedical Research Centre.

2.6 The NIHR Biomedical Research Units also undertake translational clinical research in “priority areas of high disease burden and clinical need”. They are similar in remit to the Biomedical Research Centres and aim to enable small, research groups to achieve critical mass. The NIHR funded a first round from 2008-2012, and a second round of twenty Biomedical Research Units from 2012 following open competition. There is no Biomedical Research Unit wholly focused upon children, and children are mentioned within the themes of only three, (“cardiovascular disease: optimising heart surgery in children with congenital heart defects”, University of Bristol; “musculoskeletal disease: inflammatory arthritis in children”, University of Manchester; “nutrition, diet & lifestyle: optimising nutrition to improve the health of children with chronic disorders”, University of Bristol). This may be because activity is not described explicitly; for example the Southampton Life Course Respiratory Biomedical Research Unit and the Nutrition, Diet and Lifestyle Biomedical Research Centre bring paediatricians and adult medicine researchers together in a strong multidisciplinary grouping that spans the life-course.

2.7 The UK Clinical Research Collaboration (UKCRC) was established to provide a forum to bring together the NHS, research funders, industry, regulatory bodies, Royal Colleges, patient groups and academia to facilitate and promotes high quality clinical research. The forum aims to identify opportunities and obstacles to clinical research and bring about their resolution. The UKCRC Board is made up of senior representatives from all of the partner organisations; there is currently no paediatrician on the UKCRC Board.
2.8 The UKCRC lists ten clinical research facilities in the UK that are able to support children's research. The NIHR has provided competitive opportunities for children's hospitals to bid for CRF support; however 10 of the 20 children's hospitals in the UK have no children's research facility.

2.9 Previous criticism of the research environment centred upon the immense bureaucracy and regulation (Academy of Medical Sciences, 2011), much of which arose from the poor judgement of officials charged with investigating the governance of a clinical trial in the newborn, the conduct of which was ultimately found to have been exemplary (Modi & McIntosh 2011). The UK has since made great progress in creating a research environment that ensures that the safety of research participants is safeguarded and where research is supported by regulatory processes that are nationally consistent, streamlined and proportionate. A National Research Ethics Service, funded by the Department of Health, has been introduced that provides a unified research ethics approval process for the UK and is charged with providing a service “that maximises UK competitiveness for clinical research and the return from investment in the UK”.

2.10 Research regulation is to be streamlined further under a Heath Research Authority established in 2012. The RCPCH contributed evidence to the Academy of Medical Sciences report “A new pathway for the regulation and governance of health research” (2011) that led to its establishment. We concur with the recommendations of the report, in particular “Specific populations (eg children) or the use of IMPs outside their licensed indication(s) should not be considered to be automatically ‘Level D: high risk’.” The EU Clinical Trials Regulation was designed to protect patients exposed to novel medicines. However it has resulted in a prohibitive burden being placed on many trials in children that aim only to evaluate medications or treatments already in wide clinical use. There is also continued concern about the interpretation of other regulatory requirements such as the conduct of research in emergency settings, the exclusion of children from trials, the consistency of research ethics review, the assessment of research involving children by research ethics committees that lack the necessary expertise, and disproportionate risk assessments by insurers and NHS Trust Research & Development offices for research involving children.

2.11 A principal focus of high level life sciences strategy in the UK has been on the biomedical technologies primarily as a route to disease treatment, rather than prevention, with success measured in commercial terms rather than health (Strategy for UK Life Sciences 2011). The shortcomings of this approach are evident when one considers the United States, a country that has the highest government expenditure on medical research, yet is positioned below the UK and the majority of western European countries in health ranking (Bloomberg Health Rankings 2012).

**Summary**

The last decade has seen a major focus on clinical research with sweeping re-organisations and substantial investment. In many respects recognition of the importance of clinical research and the climate for conducting research in the UK has never been better than now. There is clear need to ensure that research centred upon the early year’s benefits from these innovative national structures. Harnessing the wealth of new science tools and
technologies for early year’s research requires research groupings of clinical and non-clinical scientists working across preclinical, clinical and health services programmes.

**Recommendations**

- The success and experience of the Medicines for Children Research Network require translation into equivalent support for children’s non-medicines research

- The Health Research Authority must be more explicit of the specific needs of children within its strategy; reviews of research involving infants, children and young people should be undertaken by National Children’s Research Ethics Committees, NHS Trust Research & Development Departments, and university insurers that are competent to apply consistent criteria and a proportionate approach to risk assessment

- Support to establish multidisciplinary, cross-institutional groupings of clinical and non-clinical scientists to form virtual Biomedical Research Units and Centres, with access to diagnostic and laboratory facilities suited to children’s involvement, would optimise opportunities to harness the potential of developmental biology, children’s basic science, and life-course research

- The cardinal metrics of successful biomedical and health services research effort, indicators of population health and wellbeing should be monitored across five-year cycles with age specific data captured by research funders
3 Children’s research activity

“... healthcare is littered with the use of treatments that are based on habit or firmly held beliefs rather than evidence: treatments that often do not do any good and sometimes do substantial harm” Evans et al, in “Testing Treatments: Better Research for Better Healthcare”, Pinter &Martin 2011

“The true measure of a nation’s standing is how well it attends to its children” UNICEF 2010

3.1 National children’s research policy

In the UK, the three major funders of medical research are the NIHR, the Wellcome Trust and the Medical Research Council. No funding strategy has been identified for the NIHR. The UKCRC Health Research Classification System includes a category “reproductive health and childbirth”, but there is none for paediatric or children’s research (http://www.hrcsonline.net/pages/hrc). In the Wellcome Trust “Strategic Plan 2010-20: Extraordinary Opportunities” there is no explicit mention of children, although several areas of research clearly have some paediatric element. The Medical Research Council strategy “Research CHANGES lives: MRC Strategic Plan 2009-2014” includes a “Life course perspective” stream covering health and wellbeing from childhood to old age.

3.2 Medicines for Children Research Network and Paediatric Non-Medicines Clinical Study Group activity

The NIHR Medicines for Children Research Network (MCRN) has achieved substantial change in the landscape for UK children’s research. The number of studies in the MCRN portfolio has doubled to 300 in the two years from Sept 2009 (figure 3.1) and in the six years since it was established, approximately 25,000 children have been recruited (figure 3.2). Children’s medicine’s studies represent 3.2% of the total number listed on the NIHR Portfolio Database (August 2012) (table 3.1). The Paediatric Non-Medicines Clinical Study Group Portfolio lists 418 open/closed studies (weblink accessed 8 August 2012).

Examples of the impact of MCRN-supported studies Dr William Van’t Hoff, MCRN Co-Director

- A study of the Prevenar 13 vaccine designed to protect against 13 pneumococcal bacteria strains rather than the 7 strains covered by an earlier vaccine was shown to be more effective; it has now been licensed across the world and adopted as part of the routine vaccination programme for all children across England

- A study of H1N1 vaccines was conducted at the height of the Swine Flu pandemic and determined which vaccine dosing schedule to use with UK children; approximately 1000 children were recruited to this study over an eight week period showing how quickly studies can be conducted

- Childhood kidney diseases (predominantly congenital disorders) are very different to those of adults (largely acquired kidney disease); a study demonstrating the efficacy and safety of losartan was the first commercial study of any intervention to reduce the progression of chronic kidney disease associated with proteinuria in children

RCPCH 2012 21 of 94
- A placebo-controlled study of tocilizumab in children with systemic juvenile idiopathic arthritis led to the FDA licensing the product for use in this condition.
- The NIHR funded MCRN facilitated MAGNETiC study enabled evaluation of a novel treatment, inhaled magnesium, for severe acute asthma in children.

**Figure 3.1**  Number of MCRN studies adopted since 2006

![Graph showing number of MCRN studies adopted since 2006](image)

**Figure 3.2**  Medicines for Children Research Network total annual recruitment (excluding devolved nations) (2011-12 figures are based on half-year recruitment); (data courtesy of NIHR Medicines for Children Research Network)

![Graph showing total annual recruitment](image)
3.3 International perspectives

The European Union Clinical Trials Register provides information on clinical trials of medicinal products. A search of the register for trials in the UK completed in the ten year period commencing January 2002 identified 1700 adult studies and 220 trials in all age groups below 18 years (12.9%). Of the 220, 196 were sponsored by industry, eight by an NHS Trust, fourteen by a UK university, and one by the UK Health Protection Agency.

ClinicalTrials.gov is considered the largest and most widely used trial registry; it is estimated that it includes at least 86% of all registered trials. Bourgeois et al (2012) found that 12% of medicines trials registered with ClinicalTrials.gov between 2006 and 2011 were carried out in children. Of the 57,233 records of closed studies from 2000-2010 on ClinicalTrials.gov, 3428 (6%) involved children (Shamliyan et al 2012). Trials in young people, children and infants represent 24% of studies in the Cochrane Library Central Register of Controlled Trials (figure 3.4) (search date 11 August 2012).
Table 3.1


<table>
<thead>
<tr>
<th>Category</th>
<th>In set-up</th>
<th>Recruiting</th>
<th>Closed/Suspended</th>
<th>All</th>
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<tbody>
<tr>
<td>Cancer</td>
<td>55</td>
<td>600</td>
<td>947</td>
<td>1602</td>
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<tr>
<td>Dementias and Neurodegenerative Diseases</td>
<td>33</td>
<td>136</td>
<td>180</td>
<td>349</td>
</tr>
<tr>
<td>Diabetes</td>
<td>27</td>
<td>210</td>
<td>358</td>
<td>595</td>
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<tr>
<td>Medicines for Children</td>
<td>16</td>
<td>126</td>
<td>189</td>
<td>331</td>
</tr>
<tr>
<td>Mental Health</td>
<td>37</td>
<td>294</td>
<td>525</td>
<td>856</td>
</tr>
<tr>
<td>Stroke</td>
<td>5</td>
<td>106</td>
<td>139</td>
<td>250</td>
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<tr>
<td>Primary Care</td>
<td>52</td>
<td>369</td>
<td>679</td>
<td>1100</td>
</tr>
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<td>Blood</td>
<td>2</td>
<td>41</td>
<td>23</td>
<td>66</td>
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<tr>
<td>Cardiovascular</td>
<td>15</td>
<td>247</td>
<td>323</td>
<td>585</td>
</tr>
<tr>
<td>Genetics and Congenital Diseases</td>
<td>3</td>
<td>124</td>
<td>64</td>
<td>191</td>
</tr>
<tr>
<td>Ear</td>
<td>1</td>
<td>34</td>
<td>31</td>
<td>66</td>
</tr>
<tr>
<td>Eye</td>
<td>8</td>
<td>105</td>
<td>66</td>
<td>179</td>
</tr>
<tr>
<td>Infection</td>
<td>18</td>
<td>135</td>
<td>175</td>
<td>328</td>
</tr>
<tr>
<td>Inflammatory and Immune</td>
<td>11</td>
<td>116</td>
<td>99</td>
<td>226</td>
</tr>
<tr>
<td>Injuries and Emergencies</td>
<td>8</td>
<td>46</td>
<td>46</td>
<td>100</td>
</tr>
<tr>
<td>Metabolic and Endocrine</td>
<td>10</td>
<td>64</td>
<td>88</td>
<td>162</td>
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<tr>
<td>Musculoskeletal</td>
<td>24</td>
<td>211</td>
<td>267</td>
<td>502</td>
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<tr>
<td>Neurological</td>
<td>17</td>
<td>153</td>
<td>159</td>
<td>329</td>
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<tr>
<td>Oral and Gastrointestinal</td>
<td>13</td>
<td>182</td>
<td>136</td>
<td>331</td>
</tr>
<tr>
<td>Renal and Urogenital</td>
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<td>114</td>
<td>94</td>
<td>213</td>
</tr>
<tr>
<td>Reproductive Health</td>
<td>11</td>
<td>166</td>
<td>168</td>
<td>345</td>
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<tr>
<td>Respiratory</td>
<td>20</td>
<td>175</td>
<td>207</td>
<td>402</td>
</tr>
<tr>
<td>Skin</td>
<td>5</td>
<td>54</td>
<td>84</td>
<td>143</td>
</tr>
<tr>
<td>Generic Relevance and Cross Cutting Themes</td>
<td>42</td>
<td>519</td>
<td>614</td>
<td>1175</td>
</tr>
<tr>
<td>Total</td>
<td>438</td>
<td>4327</td>
<td>5661</td>
<td>10426</td>
</tr>
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</table>
Pandolfini and Bonati (2012) conducted a systematic search for clinical trial registries and identified clinical trials in children. They identified nine registries and found the representation of children's trials as a proportion of the total to range from 4.8%-33.3% with a weighted average of 15%. We repeated the search using the methods they described, and identified an additional eleven clinical trial registries. The results and the strategies used to search each database are shown with the data of Pandolfini and Bonati in table 3.2; with the proportion represented by children’s studies in figure 3.5. In interpreting these data caution is warranted many studies appear in multiple databases. Across the 19 trial registries from which we were able to extract data, 14.8% (range 2.4%-83.0%) of trials are aimed at children (mean weighted for size of registry). A notable exception to this is the Pan African Clinical Trials Registry in which 83% of registered studies involve children. Overall, these data suggest there has been no increase in children's registered trials over the last three years and three of the registries, including the largest, Clinicaltrials.gov, report a drop of almost 4%.

3.4 Children’s representation in global research publications

To investigate the number of children’s research publications, we conducted a systematic search in PubMed to identify every paper in the database reporting findings in humans for each year (Jan 1st - Dec 31st) over the ten year period 2002-2011. We conducted the search using PubMed filters and Boolean terms separately. The Boolean search and the search conducted using PubMed filters show strong agreement (figure 3.6). Overall studies in children appear to represent about 20% of all publications in humans. The number of papers published has increased year on year barring a decrease of 5.4% between 2010 and 2011, possibly a marker of the effect of the economic downturn on biomedical research in general. This is also reflected in the number of studies conducted with paediatric participants where a similar reduction is seen.
Figure 3.5 Percentage of registered studies conducted in children in twenty research registers across the world. Full data and details on each of the registers are provided in table 3.2.
Table 3.2  
Clinical trials currently registered in trial databases across the world, and the percentage involving participants aged <18 years (searches conducted 13/08/2012-14/08/2012)

<table>
<thead>
<tr>
<th>Register</th>
<th>Remit of Register</th>
<th>Number of paediatric trials</th>
<th>Number of trials in register</th>
<th>% of paediatric trials (2012)</th>
<th>% of paediatric trials (reported in 2009)</th>
<th>Search strategy</th>
</tr>
</thead>
<tbody>
<tr>
<td>AMGEN (<a href="http://www.amgen.com">www.amgen.com</a>)</td>
<td>Separate register now subsumed by Clinicaltrials.gov. Link on the AMGEN website allows search of clinicaltrials.gov for registered pharmaceutical trials being conducted by AMGEN</td>
<td>38</td>
<td>409</td>
<td>9.3</td>
<td>4.8</td>
<td>Clinicaltrials.gov searched using Amgen as sponsor and age limit &lt;18 years</td>
</tr>
<tr>
<td>ANZCTR (<a href="http://www.anzctr.org.au/">www.anzctr.org.au/</a>)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in Australasia</td>
<td>853</td>
<td>6916</td>
<td>12.3</td>
<td>8.5</td>
<td>Age limit &lt;18 years</td>
</tr>
<tr>
<td>Chinese Clinical trial Registry (<a href="http://www.chictr.org/en/">www.chictr.org/en/</a>)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in the China</td>
<td>109</td>
<td>2398</td>
<td>4.6</td>
<td>8.5</td>
<td>Searched the following terms individually, then excluded duplicates: child, infant, pediatric, pediatric, adolescent, toddler, baby, babies, neonate.</td>
</tr>
<tr>
<td>Clinicaltrials.gov (US) (<a href="http://clinicaltrials.gov/">http://clinicaltrials.gov/</a>)</td>
<td>Observational and interventional studies. Open to all research globally</td>
<td>29443</td>
<td>130838</td>
<td>22.5</td>
<td>26.1</td>
<td>Age limit &lt;18 years</td>
</tr>
<tr>
<td>India-Clinical Trials Registry (ctri.nic.in)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in the India</td>
<td>274</td>
<td>2889</td>
<td>9.5</td>
<td>31.4</td>
<td>Searched the following terms individually, then excluded duplicates: pediatric, pediatric, child, children, kids, adolescent, infant, newborn, toddler, baby, babies, neonate.</td>
</tr>
<tr>
<td>ISRCTN (<a href="http://www.controlled-trials.com/">www.controlled-trials.com/</a>)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in the UK</td>
<td>2712</td>
<td>10853</td>
<td>25.0</td>
<td>23.2</td>
<td>Searched using the following strategy: &quot;pediat% OR paediatr% OR adolesc% OR neonat% OR newborn% OR infant OR child% OR toddler OR babies OR baby OR kids&quot;</td>
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<tr>
<td>MRC CTU (UK) (<a href="http://www.ctu.mrc.ac.uk/study_search.aspx">www.ctu.mrc.ac.uk/study_search.aspx</a>)</td>
<td>Mainly RCTs currently being undertaken by the MRC clinical trials unit</td>
<td>21</td>
<td>216</td>
<td>9.7</td>
<td>5.9</td>
<td>Searched the following terms individually, then excluded duplicates: pediatric, pediatric, child, children, kids, adolescent, infant, newborn, toddler, baby, babies, neonate.</td>
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RCPCH 2012
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<tr>
<th>Register</th>
<th>Description</th>
<th>Number of Studies (Total)</th>
<th>Number of Studies (UK)</th>
<th>Number of Studies (European)</th>
<th>Database Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>Netherlands Trial Register</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in the Netherlands</td>
<td>425</td>
<td>3391</td>
<td>12.5</td>
<td>11.9</td>
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<tr>
<td>Sri Lanka Clinical Trials Registry</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in Sri Lanka</td>
<td>7</td>
<td>76</td>
<td>9.2</td>
<td>-</td>
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<tr>
<td>National Organisation for Rare Disorders</td>
<td>Trials currently recruiting patients with rare diseases</td>
<td>-</td>
<td>-</td>
<td>-</td>
<td>33.3</td>
</tr>
<tr>
<td>Roche</td>
<td>Registry of Phase I-IV drug trials sponsored by Roche.</td>
<td>-</td>
<td>-</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>UK pharm. industry (ABPI)</td>
<td>Was used to register trials sponsored or conducted by UK members of the ABPI.</td>
<td>-</td>
<td>-</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>CRIS (Republic of Korea)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in South Korea</td>
<td>104</td>
<td>499</td>
<td>20.8</td>
<td>-</td>
</tr>
<tr>
<td>DRKS (Germany)</td>
<td>Observational, prognosis and interventional studies. All studies are recruiting, but not necessarily hosted in Germany</td>
<td>290</td>
<td>1233</td>
<td>23.5</td>
<td>-</td>
</tr>
<tr>
<td>EudraCT (EU)</td>
<td>Observational and interventional studies. All studies have at least one investigator based in the European Union</td>
<td>2450</td>
<td>18660</td>
<td>13.1</td>
<td>-</td>
</tr>
<tr>
<td>International Federation of Pharmaceutical Manufacturers &amp; Associations (IFPMA) Clinical trials portal</td>
<td>Trials conducted by the pharmaceutical Industry. Observational and interventional studies.</td>
<td>19785</td>
<td>136137</td>
<td>14.5</td>
<td>-</td>
</tr>
</tbody>
</table>

Registers not included in Pandolfini and Bonati (2009) (5)

<table>
<thead>
<tr>
<th>Register</th>
<th>Description</th>
<th>Number of Studies (Total)</th>
<th>Number of Studies (UK)</th>
<th>Number of Studies (European)</th>
<th>Database Status</th>
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<tr>
<td>EudraCT (EU)</td>
<td>Observational and interventional studies. All studies have at least one investigator based in the European Union</td>
<td>2450</td>
<td>18660</td>
<td>13.1</td>
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<tr>
<td>International Federation of Pharmaceutical Manufacturers &amp; Associations (IFPMA) Clinical trials portal</td>
<td>Trials conducted by the pharmaceutical Industry. Observational and interventional studies.</td>
<td>19785</td>
<td>136137</td>
<td>14.5</td>
<td>-</td>
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</tbody>
</table>

Searched the following terms individually, then excluded duplicates: child, infant, paediatr, paediat, adolescent, toddler, baby, babies, neonate, newborn.

Now possible to search this registry using the following terms individually, then excluded duplicates: paediatric, pediatric, child, children, kids, adolescent, infant, newborn, toddler, baby, babies, neonate.

Database no longer appears to be functional.

Not possible to search this registry, or quantify the number of included studies.

Database no longer in existence.

“Under the age of 18” filter

pediatr* OR paediatr* OR adolesc* OR neonat* OR newborn* OR infant OR child* OR toddler OR babies OR baby OR kids

Using age range limit “under 18”
<table>
<thead>
<tr>
<th>Network</th>
<th>Type of Studies</th>
<th>Age Limit</th>
<th>Number of Studies</th>
<th>Unique Studies</th>
<th>Percentage</th>
<th>Notes</th>
</tr>
</thead>
<tbody>
<tr>
<td>IRCT (Iran) (<a href="http://www.irct.ir/">www.irct.ir/</a>)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in Iran.</td>
<td>-</td>
<td>396</td>
<td>3001</td>
<td>13.2</td>
<td>-</td>
</tr>
<tr>
<td>Pan African CTR (<a href="http://www.pactr.org/">www.pactr.org/</a>)</td>
<td>Interventional studies only. All studies are recruiting, but not necessarily hosted in Africa.</td>
<td>-</td>
<td>103</td>
<td>124</td>
<td>83.0</td>
<td>-</td>
</tr>
<tr>
<td>ReBec (Brazil) (<a href="http://www.ensaiosclinicos.gov.br">www.ensaiosclinicos.gov.br</a>)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in Brazil.</td>
<td>-</td>
<td>13</td>
<td>183</td>
<td>7.1</td>
<td>-</td>
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<tr>
<td>RPCEC (Cuba) (registroclinico.sld.cu)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in Cuba.</td>
<td>Age limit &lt; 18 years</td>
<td>10</td>
<td>418</td>
<td>2.4</td>
<td>-</td>
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<tr>
<td>UK Clinical Research Network : Portfolio Database (public.ukcrn.org.uk)</td>
<td>Observational and interventional studies. Includes studies in set up, recruiting and closed studies. To register, all studies have to meet set criteria for portfolio adoption.</td>
<td>-</td>
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<td>10496</td>
<td>4.3</td>
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</tr>
<tr>
<td>UK Clinical Trials Gateway (UK) (<a href="http://www.ukctg.nihr.ac.uk">www.ukctg.nihr.ac.uk</a>)</td>
<td>Observational and interventional studies. Hosted, but not limited to UK studies. Trials currently recruiting.</td>
<td>Age limit &lt;18 years</td>
<td>292</td>
<td>2881</td>
<td>9.9</td>
<td>-</td>
</tr>
<tr>
<td>UMIN-CTR (Japan) (<a href="http://www.umin.ac.jp/ctr/">www.umin.ac.jp/ctr/</a>)</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in Japan.</td>
<td>Age limit &lt;18 years</td>
<td>1514</td>
<td>8567</td>
<td>17.7</td>
<td>-</td>
</tr>
<tr>
<td>WHO - ICTRP</td>
<td>Observational and interventional studies. All studies are recruiting, but not necessarily hosted in Japan.</td>
<td>-</td>
<td>16574</td>
<td>179422</td>
<td>9.2</td>
<td>-</td>
</tr>
</tbody>
</table>
3.5 Types of children’s research

We searched the Cochrane Library databases to determine the extent of representation of infants, children and young people in Economic Evaluations, Health Technology Assessments, Methods Studies and Systematic Reviews (figure 3.7). In the UK children are most likely to present to primary care but a snapshot of research activity registered on the UKCRN Project Register at the end of 2008 showed minimal children’s medicines activity and just over 10 paediatric studies in primary care (fig 3.8). A search of the UK Clinical Research Network Portfolio Database shows 20 of 354 (5.6%) currently recruiting primary care studies to involve infants, children or adolescents (http://public.ukcrn.org.uk/Search/Portfolio).

Figure 3.7 Representation of infants, children and young people’s studies in the Cochrane Library Databases
3.6 Disease burden

In high income countries, non-communicable diseases are the major causes of death and disease in children, contributing 76% of disability-adjusted life years, with injuries contributing 15% and infectious diseases 9% (WHO 2012). Bourgeois et al (2012) evaluated the amount of clinical trial activity and identified considerable disparity in relation to burden of disease. In high income countries the five conditions with the highest disease burden in children were depression, schizophrenia, migraine, bipolar disorder and asthma, and in low income countries lower respiratory tract infections, diarrhoeal diseases, malaria, HIV/AIDS and depression. They identified all registered clinical medicines trials for these conditions and found that overall, 60% of the disease burden was attributable to children but only 12% of trials. The disparity was significantly greater in middle and low income countries. The disparity between burden of disease and research effort as measured by research funding is referred to as the 90:10 gap. This phrase was coined in 1990 by the Commission on Health Research for Development that evolved into the Council for Health Research on Development (http://www.cohred.org). Children are more vulnerable to the effects of this disparity.

Within specific areas of paediatric research, other limited data exists on disparity. For example in Australia, 33 (total $18 040 675) of 688 (total $355 872 646) project grants funded by the NHMRC in 2009 were related to obesity, only two of which were directed towards children ($1 283 275) with similar under-representation in previous years, despite widespread acknowledgement that paediatric obesity strongly tracks into later life and is a growing public health problem (Baur et al 2010). The top five topics of child health research attracting US National Institutes of Health funding in 2004 were “child health and development” (26%), “mental health” (13%), “diabetes” (9%), “heart and lung conditions” (9%), and “cancer” (7%) (Gitterman & Hay 2008). It is not clear what fits within the topic “child health and development” for example whether this includes public health research, but the distribution of funds does not seem to address the high burden of mental health problems in high income countries. Only three of 108 child health projects and 8995 total projects recorded in the Cordis database (http://cordis.europa.eu/home) for the European Union Framework Programmes 5, 6, and 7 (1990-2012), related to health service delivery. An EU Framework Programme 7 project, Research Inventory for Child Health in Europe (RICHE) is currently examining the gaps in European child health research. They will report to the European Commission in 2013.
3.7 Funding for children’s research

We obtained details of research funding by the NIHR, Medical Research Council, Wellcome Trust and major UK children’s research charities from published and online reports; these were checked with each organisation if not obtained from them directly. We were unable to obtain figures from the Economic and Social Research Council. We note however the £24.25M commitment by the UK Department of Business, Innovation and Skills to add to a joint investment of £4.25M from the Medical Research Council and the Economic and Social Research Council to establish a new birth cohort study in 2012. This will involve tracking 90,000 UK babies and families, and will complement a similar cohort study of 100,000 American children, the National Children’s Study, that commenced in 2007.

Total UK public and charitable research expenditure is about £2.2 billion annually of which around 5% is directed at children’s needs (Table 3.3), a proportion that has remained relatively stable over the last five years; this equates to less than £10 per child each year, compared to just under £50 per adult. Of the large number of national children’s research charities, only one, Action Medical Research, has a research spend that exceeds £1.5 million per annum (table 3.4).
<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>NIHR (Research for Patient Benefit)</td>
<td>N/A</td>
<td>1.999</td>
<td>0.225</td>
<td>5.677</td>
<td>0.9354</td>
<td>13.0%</td>
</tr>
<tr>
<td>NIHR (Programme Grants for Applied Research)</td>
<td>N/A N/A</td>
<td>0.453</td>
<td>7.4%</td>
<td>2.0263</td>
<td>1.908</td>
<td>11.8%</td>
</tr>
<tr>
<td>NIHR NETSCC funded programmes</td>
<td>N/A N/A N/A</td>
<td>N/A N/A</td>
<td>N/A N/A</td>
<td>N/A N/A</td>
<td>62.686</td>
<td>7.6%</td>
</tr>
<tr>
<td>Medical Research Council</td>
<td>573.700</td>
<td>40.500</td>
<td>7.1%</td>
<td>618.500</td>
<td>45.700</td>
<td>7.4%</td>
</tr>
<tr>
<td>Biotechnology and biological sciences research council*</td>
<td>350.900</td>
<td>0.900</td>
<td>0.3%</td>
<td>380.000</td>
<td>1.600</td>
<td>0.4%</td>
</tr>
<tr>
<td>Arthritis Research Campaign</td>
<td>N/A N/A N/A</td>
<td>27.000</td>
<td>1.046</td>
<td>3.9%</td>
<td>2.400</td>
<td>0.540</td>
</tr>
<tr>
<td>Asthma UK</td>
<td>2.818</td>
<td>0.440</td>
<td>15.6%</td>
<td>2.700</td>
<td>0.118</td>
<td>4.4%</td>
</tr>
<tr>
<td>British Heart Foundation</td>
<td>44.400</td>
<td>1.353</td>
<td>3.0%</td>
<td>94.900</td>
<td>1.962</td>
<td>2.1%</td>
</tr>
<tr>
<td>British Lung Foundation</td>
<td>1.094</td>
<td>0.198</td>
<td>18.1%</td>
<td>0.632</td>
<td>0.236</td>
<td>37.4%</td>
</tr>
<tr>
<td>The BUPA Foundation</td>
<td>0.667</td>
<td>0.271</td>
<td>40.6%</td>
<td>2.139</td>
<td>0.684</td>
<td>32.0%</td>
</tr>
<tr>
<td>Cancer Research UK</td>
<td>299.631</td>
<td>8.300</td>
<td>2.8%</td>
<td>339.187</td>
<td>8.821</td>
<td>2.6%</td>
</tr>
<tr>
<td>Diabetes UK</td>
<td>6.643</td>
<td>0.015</td>
<td>0.2%</td>
<td>6.163</td>
<td>0.740</td>
<td>12.0%</td>
</tr>
<tr>
<td>Epilepsy Research</td>
<td>0.600</td>
<td>0.060</td>
<td>9.9%</td>
<td>0.600</td>
<td>0.029</td>
<td>4.8%</td>
</tr>
<tr>
<td>Meningitis Research Foundation</td>
<td>1.443</td>
<td>0.391</td>
<td>27.1%</td>
<td>0.086</td>
<td>0.012</td>
<td>14.1%</td>
</tr>
<tr>
<td>Welcome Trust</td>
<td>333.900</td>
<td>15.400</td>
<td>4.6%</td>
<td>307.100</td>
<td>17.700</td>
<td>5.8%</td>
</tr>
<tr>
<td>Total</td>
<td>1615.795</td>
<td>67.829</td>
<td>4.2%</td>
<td>1787.148</td>
<td>79.326</td>
<td>4.4%</td>
</tr>
</tbody>
</table>

Table 3.3 UK annual total public and charitable research expenditure, and proportion for child health research, 2006-7 to 2010-11 (data prepared by Miss Karina Pall)
3.4 Illustrative children’s research charities: total research spend by year (£)

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Action Medical Research</td>
<td>1,107,794</td>
<td>1,433,709</td>
<td>979,959</td>
<td>3,013,246</td>
<td>3,016,526</td>
</tr>
<tr>
<td>Bliss</td>
<td>275,878</td>
<td>298,870</td>
<td>307,840</td>
<td>325,699</td>
<td>443,000</td>
</tr>
<tr>
<td>Child Growth Foundation</td>
<td>-</td>
<td>160,000</td>
<td>246,000</td>
<td>227,000</td>
<td>260,000</td>
</tr>
<tr>
<td>Great Ormond Street Hospital Children’s Charity</td>
<td>3,472,000</td>
<td>3,500,000</td>
<td>5,664,000</td>
<td>6,850,000</td>
<td>18,200,000</td>
</tr>
<tr>
<td>SPARKS</td>
<td>1,904,457</td>
<td>1,532,858</td>
<td>2,335,159</td>
<td>1,284,624</td>
<td>1,285,288</td>
</tr>
<tr>
<td>Wellchild</td>
<td>392,175</td>
<td>465,662</td>
<td>219,000</td>
<td>187,000</td>
<td>176,000</td>
</tr>
<tr>
<td>Juvenile Diabetes Research Foundation</td>
<td>558,000</td>
<td>859,465</td>
<td>937,532</td>
<td>1,049,000</td>
<td>1,302,792</td>
</tr>
</tbody>
</table>

3.8 Funding by industry

Industry support is almost entirely centred upon pharmaceuticals. Approximately half of MCRN studies are funded by the pharmaceutical industry and half publicly funded by UKCRC-partner organisations. The MCRN commercial portfolio is growing rapidly (Figure 3.3); of the 60 studies adopted into the MCRN Portfolio between April 2010 and March 2011, 37 (62%) were commercially-sponsored, representing a 36% increase compared to the previous year. This rate of growth is expected to continue with a predicted doubling over the next three years so that commercial studies will form the majority of the MCRN portfolio (data provided by Dr William Van’t Hoff, MCRN Co-Director).

There are no commercial studies in the Paediatric Non-Medicines Clinical Study Group Portfolio (Specialty Groups Annual Performance Management Report 2011/12 provided by Paediatric Non-Medicines Clinical Study Group Chair, Professor Anne Greenough).

3.9 International perspectives

A study of medicines trials registered in ClinicalTrials.gov with start dates between 2006 and 2011 showed that 59% of children’s trials were conducted without industry funding compared with 35% of adult trials (Bourgeois et al 2012). The Cordis research project database for the EU’s Framework Programs 5, 6, and 7 (1990-2012), shows that the European Union funded 8995 projects in medicine and health, of which 108 (1.2%) involved child health (http://cordis.europa.eu). Two projects under Framework 5 (1998-2002) were allocated to maternal/perinatal
health research, equating to just 3% of the total €64 million funding budget; in Framework 7 (2007-2013), €6.1 billion (12% of the total budget) has been allocated to translational research across six disease areas, none of which include maternal or perinatal health (Fisk & Atun 2009).

In the United States paediatric funding has remained static in absolute terms and has been reducing as a proportion of total National Institute for Health funding for a decade, to approximately a tenth of the current $30 billion budget (Gitterman & Hay 2008) (figure 3.10). Even during periods of absolute funding increases, the percentage increase for child health has been lower than for adults (Gitterman et al 2004; Hay et al 2010). In terms of National Institute for Health extramural funding (http://www.nlm.nih.gov/ep/Grants.html), 4.6% of the total budget in 2007 was awarded to paediatric departments and children’s hospitals. The other major research funder in the US, the Centers for Disease Control and Prevention awarded $35.5 million to research into birth defects and developmental disabilities in 2006, representing 0.5% of a total annual budget of $8.6 billion.

In 2006/7, 3% of the Canadian Institute of Health’s budget was spent on maternal/perinatal research (Fisk & Atun 2009). The current estimated Canadian Institute for Health Research spend on child health is $100M of about $1 billion annually (10%) (personal communication). Overall the percentage of the Australian National Health and Medical Research Council’s total budget spent on paediatric research has remained relatively constant at around 12% for the last 10 years (http://www.nhmrc.gov.au/grants/research-funding-statistics-and-data/funding-datasets/child-health). Of private funders, one of the largest is the Bill and Melinda Gates Foundation, which has spent over $15 billion on global health, with a large proportion focused on childhood vaccines, and childhood conditions such as malaria, pneumonia and diarrhoea. By 2007 they had awarded $110 million (1.4% of the total $7.9 billion budget) on new born health through the “Saving Newborn Lives” initiative (Fisk & Atun 2009), and have commitments to spend a further $1.5 billion on vaccines and $112 million on maternal and new born health (http://www.gatesfoundation.org/about/Pages/foundation-fact-sheet.aspx)

**Figure 3.9** UK Children’s and Non-Children’s Research Spend, 2006-7 to 2010-11
3.10 Studies approved by the National Research Ethics Service

The National Research Ethics Service sits within the NHS Health Research Authority and undertakes the review of approximately 7000 applications annually. In the last three years, around one in ten applications involved children (table 3.5).

Table 3.5

UK National Research Ethics Service, studies reviewed 2009-2011; (CTIMP: Clinical Trial of an Investigational Medical Product); Data courtesy of Dr Hugh Davies, NRES medical advisor

<table>
<thead>
<tr>
<th>UK figures</th>
<th>2009</th>
<th>2010</th>
<th>2011</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total number of studies reviewed</td>
<td>7783</td>
<td>7522</td>
<td>7069</td>
</tr>
<tr>
<td>Total number of studies involving children</td>
<td>783</td>
<td>867</td>
<td>896</td>
</tr>
<tr>
<td>Studies involving children as % of total studies reviewed</td>
<td>10.0%</td>
<td>11.5%</td>
<td>12.7%</td>
</tr>
<tr>
<td>Total number of CTIMP</td>
<td>915</td>
<td>755</td>
<td>783</td>
</tr>
<tr>
<td>Total number of CTIMP involving children</td>
<td>76</td>
<td>91</td>
<td>97</td>
</tr>
<tr>
<td>CTIMP involving children as % of total CTIMP</td>
<td>8.3%</td>
<td>12.0%</td>
<td>12.4%</td>
</tr>
</tbody>
</table>
3.11 Submission and acceptance of manuscripts to Archives of Disease of Childhood

The Archives of Disease of Childhood (ADC) is the journal of the Royal College of Paediatrics & Child Health. The British Medical Journal publishing group provided a data set containing details on final acceptance or rejection, and country of origin based on the address of the corresponding author (categorised as United Kingdom, European Union, Europe non-European Union, Australia and New Zealand, United States of America, Africa and Asia) of all articles submitted to ADC including the Fetal and Neonatal Edition, from 2005 to 2010. Data were anonymised to content, type of article and author names. Total submissions, submission rate as proportion of the total, and acceptance rate, were derived for each geographical region.

Between 2005 and 2010, there was an increase in submissions to ADC. Although the proportion of the total number of manuscripts submitted to ADC by UK corresponding authors fell, this was largely as a result of an increase in submissions from non-European Union countries as the number of submissions from the UK remained static during this period. Furthermore, the acceptable rate for manuscripts presented by UK corresponding authors has remained stable and is greater than that of manuscripts submitted from other countries (Table 3.6).

3.12 Publications by UK consultant paediatricians

A third of consultant level paediatricians reported having been an author on one or more peer reviewed papers published in the two year period 2009-2011 (RCPCH survey 2012). Additionally, we queried the PubMed database for the years 2007 to 2011 using the Application Programming Interface (http://www.ncbi.nlm.nih.gov/books/NBK25501/) for 3299 UK paediatric consultants listed on the RCPCH membership database. As not all consultants may publish with their middle initial, where consultants had more than one initial, 2 queries were undertaken one with and one without a middle initial (eg J Smith and JR Smith). Records for each consultant were downloaded in XML format. A list of PubMed Identifiers (PMID) for publications authored by each consultant was extracted from each author’s XML record, and the PubMed record for each PMID was downloaded (also in XML format). Duplicate records, identified from PubMed Identifiers, were removed. Records which contained ‘UK’ or ‘United Kingdom’ in the corresponding author’s institution and those where the corresponding author’s email address ended in ‘.uk’ were considered to be UK publications. Data were broken down by year (2007 through 2011 inclusive). Papers published in the top 10 biomedical journals and the top 10 paediatric journals (based on 2011 impact factor published by Thomson Reuters, Table 3.7) were identified. Papers identified as “Review” in the PubMed record were excluded. All analyses (including web-scraping the PubMed API) were undertaken with R version 2.15.1 (2012) and the XML package version 3.9-4.1 (2012).
Table 3.6 Submissions to Archives of Disease in Childhood and acceptance rates in 2005 and 2010 (Courtesy of Dr Robert Scott-Jupp, Associate Editor, ADC, Dr Damian Roland and Robyn Goodier).

<table>
<thead>
<tr>
<th></th>
<th>2005</th>
<th>2010</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total Submissions</td>
<td>1656</td>
<td>2214</td>
</tr>
<tr>
<td>UK Submissions</td>
<td>764</td>
<td>787</td>
</tr>
<tr>
<td>Rest of EU submissions*</td>
<td>302</td>
<td>484</td>
</tr>
<tr>
<td>Non-EU Europe</td>
<td>70</td>
<td>98</td>
</tr>
<tr>
<td>UK acceptance rate</td>
<td>45.6%</td>
<td>49.5%</td>
</tr>
<tr>
<td>Rest of EU acceptance rate*</td>
<td>21.5%</td>
<td>19%</td>
</tr>
<tr>
<td>Non-EU Europe acceptance rate</td>
<td>14.2%</td>
<td>10.2%</td>
</tr>
</tbody>
</table>

*EU excluding UK

A total of 488,371 PubMed records were identified as having an author with the same name as a UK consultant paediatrician (Table 3.7). Of these 61,421 originated from a corresponding author with a UK institution address; this fell to 53,540 once review articles were excluded. Of publications from paediatricians in the UK, 631 (an average of 126 per year) were published in one of the top 10 general medical journals, and 154 (approximately 30 per year) were published in one of the top 10 paediatric journals.
Table 3.7 Research publications originating in the UK and including a consultant paediatrician in the authorship, by year.

<table>
<thead>
<tr>
<th></th>
<th>2007</th>
<th>2008</th>
<th>2009</th>
<th>2010</th>
<th>2011</th>
<th>All years</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total publications†</td>
<td>71825</td>
<td>79786</td>
<td>86422</td>
<td>98880</td>
<td>112527</td>
<td>449440</td>
</tr>
<tr>
<td>Publications from UK institutions including Reviews</td>
<td>11406</td>
<td>11709</td>
<td>11952</td>
<td>12752</td>
<td>13602</td>
<td>61421</td>
</tr>
<tr>
<td>Publications‡ from UK institutions</td>
<td>9802</td>
<td>10210</td>
<td>10384</td>
<td>11191</td>
<td>11953</td>
<td>53540</td>
</tr>
<tr>
<td>Publications from UK institutions † in top 10 biomedical journals *</td>
<td>88</td>
<td>102</td>
<td>93</td>
<td>98</td>
<td>100</td>
<td>481</td>
</tr>
<tr>
<td>Publications from UK institutions † in top 10 medical journals**</td>
<td>80</td>
<td>119</td>
<td>123</td>
<td>146</td>
<td>163</td>
<td>631</td>
</tr>
<tr>
<td>Publications from UK institutions † in top 10 paediatric journals***</td>
<td>34</td>
<td>27</td>
<td>30</td>
<td>41</td>
<td>22</td>
<td>154</td>
</tr>
<tr>
<td>Total Reviews</td>
<td>7265</td>
<td>7491</td>
<td>7673</td>
<td>8167</td>
<td>8335</td>
<td>38931</td>
</tr>
<tr>
<td>Reviews from UK institutions in top 10 biomedical journals *</td>
<td>21</td>
<td>11</td>
<td>17</td>
<td>16</td>
<td>11</td>
<td>76</td>
</tr>
<tr>
<td>Reviews from UK institutions in top 10 medical journals**</td>
<td>32</td>
<td>15</td>
<td>24</td>
<td>15</td>
<td>20</td>
<td>106</td>
</tr>
<tr>
<td>Reviews from UK institutions in top 10 paediatric journals***</td>
<td>2</td>
<td>2</td>
<td>1</td>
<td>0</td>
<td>2</td>
<td>7</td>
</tr>
</tbody>
</table>

†Excludes Review Articles


Summary and recommendations

Children's representation in health research activity

The contribution of UK paediatricians to high quality international children's research is excellent, with an average of 126 peer-reviewed original research papers per year published in one of the top 10 general medical journals over the last 5 years. However the data we provide indicate that in the UK children's studies represent well below 10% of all health research, in comparison to around 15% to the international world literature. We also note a reduction in global publications, including children's research, which coincides with the economic downturn of the 2010s.

Research funding

Children's research appears to attract disproportionately little support. Children's research attracts around 5% of public and charitable biomedical research funding, equivalent to about £10 per child per annum in the UK. Only one national children's charity has a research spend that exceeds £1.5M per annum. Most effectiveness clinical trials require considerable resources, very often of the order of £1-2 million and above, emphasising the limited ability of children's charities to support substantive research on their own. Improving child health accumulates advantages throughout the life course and investment in the earliest years will reap the biggest rewards. Currently the reverse situation is true with the major proportion of health research expenditure and activity occurring in later life.

Types of research

The high proportion of pharmaceutical funded children's medicines studies in the UK reflects the aims and the success of the Medicines for Children's Research Network. In absolute terms the total number of children's medicines studies is far fewer than for adults and the number of medicines used off-label or off-licence remains unacceptably high. Stimulation of clinical trials by award of paediatric exclusivity has led to trials matching the distribution of the medicines in the adult market, but not necessarily the patterns of prescriptions in children. This suggests that market considerations, and not patient need, widely govern pharmaceutical sponsored clinical trials in children (Boots et al 2007). The fact that a medication is licensed does not necessarily ensure that it is either safe or effective; the NHS is in an ideal position to deliver pharmacokinetic studies and safety assessments in children, and monitor potential long-term side effects at low cost, facilitated by the use of routinely collected clinical data. We also identify scant children's research activity in primary care, health technology assessments, methods studies and economic evaluations, nor evidence of support for children's non-medicines research.

Relationships with industry

Historically paediatricians have often fiercely disavowed involvement with industry in particular the infant nutrition companies. Recently, perhaps stimulated by the realities of the economic downturn of the 2010s, there is suggestion of recognition that collaboration could be invaluable in fostering more rapid development of improvements in health care for children. The British Association of Perinatal Medicine is conducting a ballot of its members, the majority of who are also members of the RCPCH, regarding this issue in September 2012. A working group of the RCPCH tasked with streamlining guidance for children’s researchers and updating the RCPCH 2000 guidance for the ethical conduct of research involving children, will be addressing these and related issues and reporting early
in 2013. Key to a healthy relationship between researchers and industry is the transparency of financial arrangements, analyses of outcomes, and publication.

**Recommendations**

- We recommend consideration of ways to increase children’s medicines research especially effectiveness trials, pharmacokinetic studies and long-term safety monitoring of generics already in wide use

- The RCPCH is developing a code of conduct to guide children’s researchers working with the infant nutrition, pharmaceutical, and medical devices industries

- We recommend strategic alignment of smaller charities and large funding bodies to maximise returns from research funding
4 Research training

“The research competencies of clinical trainees have been in decline for the last few years. This has been exacerbated by a separation out of academic career structures from clinical training” Michael Quinn, Head of School, Paediatrics, South West Peninsula Deanery

“…. he (Peter Tizard) encouraged almost everybody to think research, which was very refreshing to me then and it would be today I think, because there’s so much concentration now on getting Certificates of Completion of Specialist Training rather than getting into research and having a research element to your training”

Richard WI Cooke, Emeritus Professor of Child Health, University of Liverpool; RCPCH Vice President for Science & Research, 1997-2002 (Wellcome “Witnesses to 20th Century Medicine” Series 1999)

4.1 The undergraduate years

Medical Schools have a responsibility to provide advice on career options and to foster opportunities for student doctors to explore different careers in medicine, for example during “elective” periods (General Medical Council, 2009). General Medical Council standards also state that tomorrow’s doctors should be able to critically appraise studies in the medical and scientific literature, formulate research questions, design appropriate studies to address these questions, and apply findings from the literature to help answer questions raised by specific clinical problems. Medical schools offer the opportunity for research experience to a limited number of undergraduate medical students during optional intercalated or integrated degree programmes (BSc/BA/BMedSci). Concerns have been raised around recruitment to healthcare specialities that have shorter undergraduate exposure (McManus et al 1993). In paediatrics, there has been a progressive reduction in undergraduate teaching time and clinical attachments over the last years to an average of 6.6 weeks (range 5-10 weeks) (RCPCH Academic Regional Advisors Survey 2011). Despite this, there does not appear to have been a reduction in recruitment to paediatrics which remains popular with a competition ratio of 1.84 for entry into specialty training (RCPCH 2011) and an 80% trainee satisfaction score (General Medical Council, 2011: National Training Survey 2011).

4.2 Postgraduate clinical training

After graduation, Foundation and Specialty Training Programmes build on undergraduate education to prepare doctors to deliver safe and effective practice. Some Foundation Programmes offer a four month academic rotation designed to offer the trainee protected time to explore interests in research or medical education; academic foundation programme trainees may choose to undertake experience in children’s research. We have received positive reports of these opportunities (box), but also difficulties in identifying a research supervisor, variations in support across the country, and a complicated set of regulations.

“Research in paediatrics is everything I love about medicine combined: stimulating, cutting edge, surrounded by great colleagues and extremely rewarding.”

Dr Valerie Astle, Academic Foundation Programme Trainee
4.3 Paediatric speciality training

In Paediatrics & Child Health, clinical training is provided on a “run-through” basis, in which progress from entry at Speciality Training Level 1 (ST1) to Speciality Training Level 8 (ST8) culminates in the award of a Certificate of Competition of Training (CCT). Specialty curricula require that all trainees develop their understanding of the value and purpose of medical research, and the skills to critically assess research evidence (The Gold Guide, 2010). For trainee paediatricians, Standards 18 and 25 of The Royal College of Paediatrics & Child Health Curriculum (2010) list the competencies in research required of all paediatricians. Trainee paediatricians spend time in clinical practice. They do not receive a mandatory period of research experience, although some trainees take up opportunities to acquire this through a structured programme such as that provided in some Masters courses or by taking time Out of Programme for Research (OOPR). Exposing trainees to research in the early part of their training increases their interest and their chances of success in securing competitive funding later in their careers.

4.4 The Integrated Academic Training Pathway

The Integrated Academic Training Pathway was established following the recommendations of a joint sub-committee of the UK Clinical Research Collaboration and the NHS, chaired by Professor Sir Mark Walport (“Medically and Dentally qualified academic staff: Recommendations for training the researchers and educators of the future” Academic Careers Sub-Committee of MMC, 2005). Trainees, who may or may not have had a prior appointment to an academic foundation post, apply in open competition for an Academic Clinical Fellowship that combines 75% clinical and 25% research training. The goal for the academic clinical fellow during this period is to gather preliminary data, skills and knowledge to enable application for a Research Training Fellowship leading to a PhD, or research MD, and subsequently an Academic Clinical Lectureship. This represents a clinical academic career pathway for those who wish to train for a research career. The development of this pathway, where clinical research training is offered by leading clinical academics in partnership with Universities, NHS Trusts and Postgraduate Deaneries, has provided structure for clinical academic training but requires motivation, career guidance, research training posts, and suitable research supervisors. The Integrated Academic Training Pathway provides the bedrock for medical research training in for most specialities and has led to clarity for those who make a decision early in their training that they wish to pursue a clinical academic career. There are separate but similar schemes in the devolved UK nations.

Around 200-250 Academic clinical Fellowships have been appointed each year since 2006 and paediatrics has fared well, being awarded approximately 10% of all posts each year (Table 4.1). It is too early to see the full impact of this investment in structured academic training but preliminary figures for the Academic Clinical Fellowships awarded to Paediatrics in 2006, 2007 and 2008, show 29 trainees (60%) to be continuing in research training (including 4 MRC, 7 Wellcome and 3 NIHR fellowships), 3 have moved abroad and 13 have returned to clinical training; for remaining the outcome is as yet unknown (data courtesy of NIHR Trainees Coordinating Centre).
Table 4.1
National Institute for Health Research Academic Clinical Fellowship (ACF) awards, total awards and awards to paediatricians, 2006 to 2011 (data courtesy of NIHR Trainees Coordinating Centre)

<table>
<thead>
<tr>
<th></th>
<th>2006</th>
<th>2007</th>
<th>2008</th>
<th>2009</th>
<th>2010</th>
<th>2011</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Paediatric ACF posts</td>
<td>14</td>
<td>10</td>
<td>23</td>
<td>23</td>
<td>35</td>
<td>19</td>
<td>124</td>
</tr>
<tr>
<td>Total ACF Posts</td>
<td>138</td>
<td>181</td>
<td>237</td>
<td>256</td>
<td>254</td>
<td>252</td>
<td>1318</td>
</tr>
</tbody>
</table>

| Percentage awarded to Paediatrics | 10.1% | 5.5% | 9.7% | 9.0% | 13.8% | 7.7% | 9.4% |

“*I have found the ACF to be a valuable opportunity to develop my academic skills. Meeting with fellow academics challenges and encourages me*”
Dr Andrew McArdle, Academic Clinical Fellow

“It was a bit tricky dovetailing my academic interests, clinical work and membership preparation, but I found my clinical placements were consistently very supportive and helpful in allowing me study leave. For me the best points of the ACF have been the solid block of time it gave me to devote to research”
Dr Felicity Fitzgerald, Academic Clinical Fellow

Figure 4.1 Training for a Career in Research for Children.
Representative examples of training pathways for: A) an ACF starting at ST1 in the Integrated Academic Training Route B) an ACF starting at ST1 later also appointed to an ACL post in the Integrated Academic Training Route C) an trainee undertaking a PhD in time Out-of-Programme for Research (OOPR) by individualised training route (example starting at ST4) D) Standard Clinical Training Route

Boxes and numbers: training year; Open, clinical training; Closed, academic/research training; ACF: Academic Clinical Fellow; ACL: Academic Clinical Lecturer; ST: Speciality Training Year. Where a PhD is undertaken, ST6 is omitted, as one year of the time spent out of a specialty training programme for research purposes is recognised towards the award of a CCT (RCPCH”GOLD GUIDE” 2010)

4.5 Other academic training routes

Career inclinations may take time to crystallise. For example, one quarter of those working in clinical Paediatrics ten years after their graduation from Medical School in 1993-1996, had not identified Paediatrics as their speciality of choice in their first year after qualifying (Goldacre et al 2010). Whilst this has remained stable for cohorts of medical graduates from 1974-1996 despite the change in working practices towards more intense and resident working, the introduction of run-through training in Paediatrics following “Modernising Medical Careers “ (2004) might be expected to increase this further.

Although intended to be flexible, in many Postgraduate Deaneries entry to the Integrated Academic Training Pathway has not been possible later in clinical training (Figure 4.1). This disadvantages those who develop an interest in research at a later stage. These clinically experienced trainees bring the wealth of clinical and life experiences to the research they undertake and may make excellent, even better, clinical researchers. Yet they often describe difficulties in accessing information, finding an academic supervisor, and obtaining funding and approval to go Out of Programme for Research whilst maintaining their clinical speciality training number. Speciality training programmes in principle permit trainees to undertake a period of research during their clinical ‘run-through’ training but prolonged notice periods and difficulties in accessing opportunities continue to frustrate. Those who do succeed in surmounting these challenges find that they are not eligible to participate in the networking and educational opportunities for trainees in the NIHR Integrated Academic Training Pathway.

Training and experience in certain sub-specialities such as paediatric psychiatry, cardiology and haematology takes place at the latter stages of the training pathway. This has also led to difficulty in relation to the Integrated Academic Training Pathway in which Academic Clinical Fellowships are meant to be appointed earlier at ST1-3 level (Garralda 2011).

“It is entirely possible to develop a PhD project and apply for funding without being in an ACF post and there are even some advantages.”
Dr Alasdair Bamford, NIHR Research Training Fellow

“ACFs/ ACLs are still not the only way in”
D Hoong Wei Gan, Specialty Registrar OOPR
The temptation with the current career structure is to get on your track and continue to the end. If you have an interest [in research], then I would suggest looking sideways at other opportunities.”

Dr Daniel Hawcutt, Academic Clinical Lecturer

The RCPCH Education & Training Support Centre undertook a survey of all UK Postgraduate Deaneries in 2011. All nineteen distinct Postgraduate Deaneries responded, notifying 159 (4.4%) trainees holding National Training Numbers in Paediatrics at the time of the survey who were “Out Of Programme for Research” or in an integrated clinical academic training post, either an Academic Clinical Fellowship or an Academic Clinical Lectureship. In adult medicine, almost 20% of specialist registrars and speciality trainees undertake a dedicated period of research training after core training (Royal College of Physicians Specialist Registrar Survey 2011), with as many as 23% undertaking a higher research degrees (MD or PhD) in some sub-specialities such as rheumatology (Gompels et al 2011). In paediatrics, attainment of a PhD or research MD increases with year of training from 2.4% of those in their first year of speciality training to about 9% at completion (RCPCH Trainees Survey 2012). Of note is that approximately 50% of academic trainees wish to return to full time clinical training before going back into academic medicine (Royal College of Physicians Specialist Registrar Survey 2011).

“Designing, planning and carrying out my research project has convinced me of the value of a period of research to any clinician, whether or not they wish to pursue an academic career. Self-direction, critical and original thinking, creative problem solving and developing an evidence base for practice are skills vital in an academic environment, but equally transferable to clinical work”.

Dr Sarah Eisen, Wellcome Trust Research Training Fellow, ACF 2007-10

“Although I am not in an academic post, I am involved in research on a daily basis. My approach to clinical medicine has been developed as a result of my research training. Experience of research has made me a more knowledgeable, more rounded clinician and has improved the way I practice, teach and learn medicine.”

Dr Stephen Wardle MD, Consultant

4.6 Trainee confidence in core research skills

The pilot phase of the new RCPCH ST7 assessment (to be introduced as ‘START’ in 2012) revealed that paediatric trainees feel poorly prepared in relation to core research standards (Modi, 2011).

From November 2011 to February 2012 the RCPCH Trainees’ Committee undertook a national survey. For the first time, in response to the “Turning the Tide” initiative, this included a number of questions relating to research experience. A total of 2037 UK paediatric trainees responded to the survey, representing a response rate of approximately 56% (Table 4.2).
Table 4.2  RCPCH Trainees’ Survey 2012

<table>
<thead>
<tr>
<th></th>
<th>All Trainees (n=2037)</th>
<th>Trainees* at ST7+ (n=388)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Had not achieved curriculum competencies in interpretation and conduct of children's research</td>
<td>26.7%</td>
<td>11.5%</td>
</tr>
<tr>
<td>Trainees feel competent to:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Literature search and critically appraise</td>
<td>81.4%</td>
<td>90.4%</td>
</tr>
<tr>
<td>Consent patients for approved research studies</td>
<td>56.6%</td>
<td>85.4%</td>
</tr>
<tr>
<td>Develop a research question and design a research study</td>
<td>35.1%</td>
<td>57.4%</td>
</tr>
<tr>
<td>Disseminate research findings by oral presentation</td>
<td>33.7%</td>
<td>49.3%</td>
</tr>
<tr>
<td>Disseminate research findings by preparing a manuscript for publication as a peer-review paper</td>
<td>79%</td>
<td>89%</td>
</tr>
<tr>
<td>Proportion with a higher research degree (PhD)</td>
<td>3.4%</td>
<td>8.8%</td>
</tr>
</tbody>
</table>

*Trainees in the final 2 years of training in paediatrics

Key results of RCPCH Trainees Survey 2012

- A quarter of respondents overall, and 11.5% within the final 2 years before award of the Certificate of Completion of Training (ST7 and above), report that they had not had the opportunity to gain research competencies relevant to their stage of training with only 6% reporting having had access to structured research training
- Almost half report not feeling competent to take informed consent for recruitment to research studies
- One third of respondents feel they understand statistical tests used in medicine or feel competent to develop a research question relative to their stage of training
- 10% within the final 2 years of training do not feel competent in critical appraisal of research literature, the key skill in the practice of evidence based medicine
- Only around one third of trainees were aware of how to access advice on academic training and this was variable across the regions of the United Kingdom (with a range of 7-70% between the UK Postgraduate Deaneries)
- 77% of respondents reported that they would be either likely or very likely to spend a short voluntary period of up to 6 months attached to a paediatric research group to gain research experience if this was offered as part of their training programme
The survey provides clear evidence (box) that trainees feel insufficiently prepared for research involvement in many basic respects. Nevertheless, many trainee paediatricians work with seniors to undertake, present and publish case reports and research studies; for example, one in five trainees in the London Deanery in a cross-sectional study in 2011 have published at least one case report or paper (C Fertleman, personal communication). In some disciplines, notably surgery, trainees have taken matters into their own hands by developing their own networks to promote trainee involvement in research and multicentre study participation (Bartlett et al 2012).

4.7 Trainee forums

The RCPCH Annual Conference offers opportunity for paediatric trainees to present original research. Submissions are selected by peer-review and scored blind to status (trainee or consultant level). In 2010, 49% (176/356) of submissions by trainees were accepted for presentation; in 2012, this had fallen to 23% (100/434). The number of slots for presentations had risen and those available for trainees to present had not reduced. Therefore, one interpretation is that the quality of abstracts submitted by trainees has fallen.

Scottish and Welsh Paediatric Societies meet biannually attended by a high proportion of paediatric trainees in their respective devolved nations. These meetings provide forums for trainees and consultants for Continuing Professional Development and presentation of cases and research relevant to child health. Whilst advocacy and contribution to research are not considered their direct responsibility, bursaries are made available for those wishing to present work undertaken in these devolved nations at international meetings.

Research societies can help raise awareness and enthusiasm; for example, The Neonatal Society [http://www.neonatalsociety.ac.uk/], has three meetings a year and offers bursaries to support trainee involvement in research during elective placements, and awards prizes for trainee and student research presentations.

4.8 Research opportunities for clinical track trainees

There are examples of innovative approaches that are helping to provide short-term opportunities for research experience. Northumberland Tyne and Wear Comprehensive Local Research Network have developed new Clinical Research Associate posts for clinicians in training. These provide a year's salary with funding for successful candidates to complete the first taught year of the MClinRes course, leading to a certificate or Diploma in Clinical Research. These are viewed as entry level research posts, and are appointed by open competition. The Clinical Research Associates are assigned to a supervisor and gain experience by working on NIHR Portfolio studies. They complete training in research Good Clinical Practice, consent taking and study management. The Northern Deanery has been involved in arranging for Out of Programme Approval for Education (OOPE) for these posts; four of seven posts appointed in 2012/13 were awarded to paediatric trainees.

Some trainees may wish to take up opportunities offered through programmes such as the University of Glasgow Certificate in Child Health, primarily designed for post MRCPCH paediatric trainees in ST years 3, 4 or 5 that includes developing
skills in problem solving, experimental design, evaluation and critical interpretation of clinical data, literature searches, and team working, and lays the foundation for students wishing to pursue further studies leading to a Diploma or MSc in Child Health, and other Child Health MSc programmes. Training in advanced research skills such as provided in Masters of Research (MRes) programmes are also available, and there is increasing availability of high quality resource in the international literature (Hartling et al 2012).

Summary and recommendations

Fostering a spirit of enquiry

The love of science and the thrill of exploration that are part of the fabric of medical school entrants must be fostered and developed throughout their undergraduate careers and through the years of clinical training. Scholarship and the spirit of enquiry are essential components of a medical career. Trainees come most into contact with consultant paediatricians but as we show in section 5 the number that have had experience in research is falling, thus perpetuating a cycle of disengagement. A significant worry is the, perhaps infrequent, but no less destructive and lingering perception that not all doctors need to be concerned with research, and a dismissal of its value. Perhaps the greatest challenge for the RCPCH is to effect a change in culture and eliminate these perceptions so that clinical practice and research are once again seen as integral to all paediatricians.

Medical students

The likelihood of significant experience of paediatric research during the short period of undergraduate paediatric training is small. Instilling an enthusiasm for children's research requires students to be taught by leading clinical academic paediatricians, as well senior clinicians but, as we discuss in Section 6, the number of the former in now very small. There is better opportunity for paediatric research experience during integrated and intercalated degree programmes and during student electives. Other opportunities include innovations such as L-Smart, a University of Leicester medical student academic teaching programme and the University of Nottingham summer research school.

Paediatric trainees

The RCPCH trainee survey confirmed major problems in relation to research training and experience for paediatricians. There is limited and in many cases non-existent formal training in research techniques, regulation and governance for paediatric trainees. If the care of sick children and the prevention of ill health are to be improved, the need for research and the acquisition of skills appropriate to the planned career must be embraced by trainees – the consultant paediatricians of the future - and employers alike. Some trainees will seek a clinical academic career in which time is spent both in patient care and research, whilst others will pursue a wholly, or predominantly, clinical career. For both, the acquisition of core competencies in children's research is essential.

Trainees require good grounding in the tools of research even if these are to be used for non-research purposes such as high quality audits, surveys and other health service evaluations. When performed to methodologically rigorous standards, these have substantial potential to improve both trainees' skills and, importantly, patient care. All
trainees must also understand the organisation, regulation and governance of research in the UK, and acquire key skills such as that of obtaining assent and informed consent.

**Flexibility in training**

Trainees who wish to explore options, perhaps undertaking a short period in research, or seeking experience or later in their training, all too often find themselves frustrated by inflexibility of the current paediatric clinical training pathway. It is essential that there are a variety of entry points and routes for trainees to acquire research training (Thompson & Evans, 2009) and there must be flexibility to move between so-called “academic” and “clinical” training tracks. We appreciate the pressures to fill clinical rotations but Postgraduate Deaneries and/or equivalent structures have an obligation to ensure the most appropriate opportunities for trainees. The RCPCH has a clear commitment to high quality training and the need to move progressively towards consultant delivered services so that training does not take second place to service delivery.

RCPCH Academic and Clinical Regional Advisors, and Postgraduate Deaneries must ensure a consistent, flexible and appropriately timely approach to considering applications for periods Out-of-Programme for Research in line with current recommendations that “Trainees should be encouraged and facilitated to undertake research where they have an interest in doing so” (A Reference Guide for Postgraduate Specialty Training in the UK: The Gold Guide 4th Ed. June 2010, Section 6.78). Once granted the expectation should be that Postgraduate Deaneries support taking up OOPR at the earliest and most appropriate opportunity and facilitate within or between Deanery transfers should this be necessary, as for example with research training in a “niche’ sub-speciality. Flexibility is also required of funders to agree activation of research training awards coordinated with application for OOPR approval.

**Guidance**

Medical students and paediatric trainees should be made aware of the attractions of a research-active career regardless of whether their intention is to pursue an academic or clinical training track. They require clear advice in line with the recommendations of the Academic Careers Sub-Committee of MMC (2005) and signposting to clinical academic mentors who can advise on how to pursue a career in research. Improved understanding of the “research pathway” could help could play an important role in helping demystify and familiarise medical students in these core areas. The members of the RCPCH Commission on Child Health Research have contributed to the development of a comprehensive Guide to Training in Child Health Research. This has been made widely available through Heads of School, Academic Regional Advisors, Regional Advisors, College Tutors and the RCPCH website.

**Assessing research skills**

Ensuring that achieving the research competencies set out in the RCPCH Curriculum Sections 18 and 25 is a key role for all RCPCH Educational and Clinical Supervisors. A higher degree should not be seen as the only measure of research training. Participation in a Masters of Research (MRes) programme or MSc in Paediatrics/Child Health should be encouraged as is already the case in some postgraduate deaneries. Other objective criteria for research experience are set out in the research competencies section of the e-portfolio (Box). Formal assessment of research competencies takes place in the MRCPCH examinations and in START (Specialty Trainee Assessment of Readiness for Tenure). In
order to facilitate the recording and presentation of evidence at the Annual Review of Competency Progression the RCPCH has developed a flexible research competencies section integrated into the existing training e-portfolio. This may be used by all paediatric trainees, regardless of whether they are on clinical or academic tracks and avoids duplication in parallel systems. This offers flexibility for all paediatricians in training to record their competencies in child health research regardless of the timing of its acquisition whilst also allowing presentation of evidence of enhanced training in research for children in line with the Guidance of the Academy of Medical Sciences (2011).

Providing opportunity

There are limited opportunities for short-term research experience that are primarily directed at clinical track trainees. This experience, similar in many ways to that gained by medical students undertaking “intercalated” BSc/BA/BMedSci degrees, should map to the RCPCH curriculum, must be supported with supervisor reports in the integrated e-portfolio, and should be assessed at the Annual Review of Competency Progression. This requires identification of potential research active supervisors, funding, and the support of Postgraduate Deaneries or Local Education Training Boards responsible for the training of the Paediatricians of the future. Training should be offered in a range of formats that cover requirements most relevant to the majority of clinicians. The RCPCH is establishing a new Education Centre that will help deliver this agenda, and a Research Training Committee to provide oversight.

Recommendations

- Opportunities to join a clinical or non-clinical child health research group should be fostered at undergraduate and postgraduate level

- We recommend that optional attachments to an established research group for short periods of 3 months to one year, awarded in open competition, are incorporated into paediatric clinical training programmes

- We recommend RCPCH Academic Regional Advisors include within their remit responsibility for signposting medical students and paediatric trainees to child health researchers who are able to provide research opportunity and supervision, and provide clear, consistent guidance on routes into research, and requirements for approval to take time out of clinical training for research

- While current UK regulation requires training in research ‘Good Clinical Practice’ for clinicians involved in recruiting to clinical trials, this should be strongly encouraged for all trainees; we do not recommend that obtaining a Masters or higher research degree should be mandatory, nor do we consider this a necessary requirement for a research career

- We recommend that progress in attaining competencies in child health research form part of the Annual Review of Competency Progression for all paediatric trainees
Criteria for assessment of research experience/competencies

RCPCH e-portfolio research skills log
- Approaching for study consent
- Gaining study consent
- Randomising for study treatment
- Recording study data for a research study
- Making a research database
- Undertaking study data analysis
- Designing and displaying data graphically
- Designing a poster of research data
- Making a research presentation
- Laboratory technique
- Managing a research study

RCPCH e-portfolio Research Training Assessment
- Achieving Research Competencies in the Curriculum (Assessment Standard 25)
- Progress with examinations (e.g. MRCPCH, PhD, Research MD, Research MSc as relevant)
- Generic research skills
- Research methods
- Research Good Clinical Practice training
- Consenting participants for research studies
- Critical appraisal of published research
- Research governance
- Research funding applications
- Undertaking research/research study progress
- Presentations of research
- Supervising research
- Research publications
- Progress of personal research programme
- Teaching
5 Research and the NHS

“The Government is determined to make the UK the best place in the world for health research, development and innovation” Foreword, “Best research for best health”

5.1 Best research for best health

The UK Department of Health report, “Best research for best health” issued in 2006, set out a strategy to place patients at the heart of clinical research (Department of Health 2006). The NHS was to be established as an internationally competitive centre of research excellence that would attract, develop and retain able research professionals. Research would be commissioned that would focus on improving health and care and strengthening and streamlining systems for research management and governance. Within five years there was to be a thriving research culture within the NHS, increased opportunity for patients to take part in multicentre studies and preventive strategies, increased industry investment in clinical research and the development of improved information technologies to support research. This was a visionary agenda with great potential to serve infants, children and young people well.

5.2 The possibilities

There are approximately 13 million infants, children and young people in the UK, comprising one fifth of the total population. Up to half of all infants under 12 months of age and one quarter of older children will attend a National Health Service Accident & Emergency Department; one in 11 children will be referred to a hospital outpatient clinic; one in 10 children will be admitted to hospital and one in 1,000 children will require intensive care; one in 10 newborn babies require admission to a neonatal unit and of these about 2% will need intensive care. Children aged 0-14 years account for 2 million (12%) of a total of approximately 17 million Finished Consultant Episodes in UK hospitals annually (HES Online), yet each year less than 2.5% of episodes involve recruitment into clinical research studies. The exemplar speciality for recruitment and close integration of research with clinical care is paediatric cancer. In most developed countries, patients with cancer from infancy to the age of 15 years benefit from highly coordinated specialised children’s services with approximately 70% of all children enrolled into a clinical trial. Approximately three-quarters of children with cancer now survive. In contrast such co-ordination is lacking for older teenagers and young adults, and in this group survival rates have not shown the same improvement (Fern & Whelan 2010).

5.3 A core NHS role

The UK Government in its White Paper, "Equity and excellence: Liberating the NHS" (2010) made extensive reference to the importance of research evidence as central to the NHS, noting “The Government is committed to the promotion and conduct of research as a core NHS role” (para 3.16), "The Department is committed to evidence-based policy-making and a culture of evaluation and learning" (para 1.23), “The forthcoming Health Bill will support the creation of a new Public Health
Service...including an increased emphasis on research, analysis and evaluation” (para 4.15) and emphasising the importance of patient involvement in research stating "This [shared decision-making] is equally true of the partnership between patients and clinicians in research, where those institutions with strong participation in clinical trials tend to have better outcomes” (para 2.3).

5.4 The Health and Social Care Act 2012

During the passage of the Health and Social Care Act 2012, the UK Government introduced a number of measures designed to increase biomedical research in the NHS further. Organisational structures within the new NHS including the Clinical Commissioning Groups, National Commissioning Board and Public Health England, and the Secretary of State for Health, have a statutory duty to engage in and promote research. The Government has also acknowledged on multiple occasions the significant contributions that health research can make to the UK.

5.5 A research culture

A National Institute of Health Research survey published on International Clinical Trials Day on 21 May 2012 shows that much more is needed to create a research culture within the NHS. Only a fifth (21%) of people surveyed are aware that carrying out research is a key activity for the NHS, though four-fifths (82%) consider it important for the NHS to offer opportunities to take part in research (NIHR survey, 2012). In a prior survey of healthcare professionals, carried out in 2011 for the National Institute for Health Research Clinical Research Network, 61% of respondents said that research was peripheral in their NHS Trust, and only 38% felt that research was embedded in planning and performance at board level.

5.6 NHS processes and children’s research

A characteristic of clinical trials in infancy and childhood is the need for long-term follow up to evaluate late outcomes and establish safety. Children at high risk of later impairments also require skilled follow-up as part of on-going care. At present arrangements for follow-up evaluations of infants and children are often duplicated for clinical and research purposes. Similarly data collection is often repetitive with bespoke systems and processes established for each NHS purpose or research project when capture once to a high standard would reduce costs, improve efficiency and achieve higher quality data. This is has been shown to be possible in neonatal specialised care, where coverage of data capture is close on 100% across England and Wales. Responsibility lies with clinical teams, and has resulted in extremely high levels of completeness and quality. These data are used to create a National Neonatal Research Database that serves multiple purposes across NHS needs and research (Spencer and Modi 2012).

5.7 The European Union Working Time Directive

This was implemented into UK legislation in 2009. The Directive limits the time a doctor may work to 48 hours per week; the inevitable consequence has been a
substantial shortfall in the availability of trainees to fill clinical rotas, increasing the pressure upon consultants as to maintain the clinical service and diminishing the opportunity for activities not directly related to clinical care, foremost of which is research involvement. The limits placed on doctor's working time has also resulted in a 95% increase in the number of NHS paediatric consultants from 1605 to 3122 Full Time Equivalents between 2000 and 2010. Over the same time period the consultant level academic paediatric workforce increased by 4.9%, from 180 to 189 FTE. In General Medicine the NHS consultant workforce and the academic consultant level workforce increased by 59% and 58% respectively (Medical Schools Council 2011).

5.8 The consultant contract

The period of time that saw the NHS placed centre-stage for patient focused research also saw the introduction of the “new” consultant contract and the consultant “job plan”. This sets out on an hour by hour basis the activities that an NHS consultant is contracted to undertake and that the employing Trust must remunerate, in the 4 domains of direct clinical care, teaching, administrative duties and research. Today a NHS consultant is unlikely to be able to engage in appreciable research activity unless this is clearly identified Programmed Activity (PA) in his or her job plan and is fully funded.

The RCPCH conducted a personal census of consultant and Staff, Specialty and Associate Specialist Grade paediatricians in 2012. Responses were received from 2280 paediatricians, representing 67% of the total consultant level workforce. Of respondents, 66% of consultant level paediatricians have no allocated Programmed Activities (a PA represents 4 hours per week) for research; 26% have one PA or less, 7% have 1.5 to 5 PAs; only 1%, all of whom hold academic appointments, report receiving more than 5 research PAs. Of the total reported paediatric PAs delivered in the NHS, 4.7% (1013/21581) were for research. This in contrast to the Royal College of Physicians census in 2010 (with a return rate of approximately 50%) in which they report that 82% of respondents have at least some time allocated for academic activities each week, and that an average of 6.5% of all contracted PAs in the adult medical specialities are for research.

5.9 Paediatric consultant research experience

The average age of first consultant among UK graduates is 35 years. Of 1497 respondents reporting having qualified from a UK medical school the proportion in age bands <40, 40-49, 50-59, and >60 years, with the research degree MD or PhD, were 11.5%, 29%, 34% and 35% respectively. The reduction in the proportion of consultants holding a higher research degree in the younger age groups is in keeping with the widespread perception that research experience is declining in the consultant paediatric workforce.

5.10 Funding to support paediatric consultant involvement in research

The landscape has changed substantially since the creation of the National Institute for Health Research and considerable funding is now potentially available for
clinical research. It is important that Consultant Paediatricians are aware of the several potential sources of funding for Programmed Activities for research that are available. The approach to be adopted depends on the nature and ultimate goal of the research involvement, and local circumstances. There are also differences between the four UK nations. Support can be obtained directly through research grants. In England support from the National Institute of Health Research can also be obtained through Local Research Networks, Research Capability Funding (previously Flexibility and Sustainability Funding), Biomedical Research Centres and Biomedical Research Units (box). Specific arrangements differ in Wales, Scotland and Northern Ireland. In Wales, for example, from 2010 there have been applications through Universities for around 50 Senior Fellowships for up to 4 programmed activities per week to support NHS staff for up to three years. In Wales from 2010 there have been applications through Universities for around 50 Senior Fellowships to support NHS staff for up to 4 programmed activities for up to three years. Paediatrics secured three Fellowships, with two NHS paediatric consultants and a paediatric clinical psychologist each receiving funding for 2 programmed activities each.

**Sources of funding for consultant Programmed Activities for research**

**(Dr Mark Turner)**

**Direct support from grants**

This is the most straightforward. Consultant paediatricians and other career grade staff should ensure the time they will spend on research as lead or co-investigator is included as a cost in the grant application. This will need to be approved by their Trust through the Research & Development Office. It is also advisable to get agreement from the Trust in advance about how these funds will be used should the application be successful such as the mechanism for using the money to back-fill the time taken out of clinical duties by the investigator, or if this is not possible, whether the number of remunerated programmed activities will be increased for the duration of the grant.

**Local Research Network Funding**

The two types of local NIHR research network, the Comprehensive Local Research Networks and the Medicines for Children Research Network Local Research Networks have a responsibility and funding to support NIHR portfolio studies to recruit and deliver successfully. Some networks will additionally choose to fund “research champions” or “research leads” to promote research recruitment. What this involves in practice varies between networks. All NIHR networks, whether national or local, are managed by performance and therefore success in attracting funding for paediatric consultant involvement to support studies is likely to be greater if there is a need to improve recruitment to children's studies. Networks may prefer to appoint research nurses to recruit and support studies, so the added value of medical involvement will need to be justified. The amount of funding allocated to support paediatric studies also varies considerably between networks. Nonetheless this option is an ideal way for NHS Trusts to secure funding for Paediatric Consultant programmed activities to contribute to the national research agenda. Trusts, through their Research & Development Director, should discuss with
the Paediatric Speciality Group member of their CLRN or the MCRN Local Research Network Director, if located in a MCRN network

http://www.crncc.nihr.ac.uk/about_us/ccrn/specialty/paed (last accessed 3 January 2012).


Research Capability Funding

Research Capability Funding (formerly Flexibility and Sustainability Funding) is awarded to NHS Trusts according to their contribution to NIHR portfolio studies. Trusts can use Research Capability Funding for short-term support for staff who support NIHR portfolio activity or who wish to develop a grant application that has a strong chance of success and which fits with the Trust’s research strategy. This option should be discussed with the Trust R&D Director.

Biomedical Research Centres and Biomedical Research Units

Biomedical Research Centres (BRC) and Biomedical Research Units (BRU) were established in 2008, with a second wave commencing in 2012. There are 11 theme based BRC and 20 BRU focused on specific disease topics such as gastrointestinal disease. Some BRC and BRU will have a degree of flexibility about how their funds are allocated; in others funding decisions will have been made in advance. In any case support for consultant programmed activities will be tied tightly to the BRC/BRU theme or strategy. If you feel you can contribute discuss this directly with the BRC/BRU lead or with your Trust Research &Development Director.

http://www.nihr.ac.uk/infrastructure/Pages/infrastructure_biomedical_research_centres.aspx

http://www.nihr.ac.uk/infrastructure/Pages/infrastructure_biomedical_research_units.aspx

Personal profile

Dr Sabita Uthaya is a consultant neonatologist with Chelsea & Westminster NHS Foundation Trust. Following award of the Certificate of Completion of Training Sabita self-funded a period of research that lead to the degree MD from Imperial College London. Supported by her academic supervisor, Sabita was encouraged to apply to join a National Research Ethics Committee, and to develop her research interests. She now chairs the Research Ethics Committee and in 2009 she was awarded funding through the MRC/NIHR Efficacy & Mechanism programme as Lead Investigator of a randomised controlled trial of parenteral nutrition in preterm babies. This included personal support for 2 Programmed Activities. In 2012 she was awarded 2 additional programmed activities for one year, through the Trust’s CLRN allocation, to enable her to develop further grant applications. Her case illustrates how research active NHS paediatricians can assume leadership roles in research, and secure personal support through a variety of funding routes.
Personal profile

Dr Heike Rabe is a consultant neonatologist with a long-standing interest in infant haemodynamics. From 2009 she was supported for two programmed activities a week from the Flexibility and Sustainability Funding allocation of her employer, Brighton and Sussex University Hospitals NHS Trust. This helped her develop grant applications relating to neonatal inotrope use. She now leads a 6 million Euro project (NeoCirculation) funded by EC FP7 through which she will carry out studies to support a Paediatric Use Marketing Authorisation for dobutamine, and develop an international consensus definition of neonatal shock. Heike moved into an academic position with Brighton & Sussex Medical School at the start of 2012. Her case demonstrates how NIHR funding can be used to support paediatricians develop world-class research and move from research active NHS roles into academic positions.

Personal profile

Dr Alison Leaf is a consultant neonatologist with University Hospital Southampton NHS Foundation Trust. Alison undertook doctoral research before becoming a full time NHS Consultant. She continued to develop an interest in neonatal nutrition which led to her leading a trial funded by Action Medical Research, examining early feeding regimens in compromised preterm infants. Recently Alison moved to a position as a neonatologist with protected research time supported by the Biomedical Research Centre in Nutrition at Southampton. Her case illustrates how new opportunities for research-active NHS posts have become available as a consequence of the foundation of Biomedical Research Units and Centres.

Personal profile

Dr Catherine Tuffrey was appointed community paediatric consultant with Solent NHS Trust at the end of 2010, having previously undertaken a post-CCT PhD. The Research & Development Director of the acute Trust in Portsmouth offered her the opportunity of one Programmed Activity a week funded from local CLRN monies, to support general paediatric research as many of the consultants had no research experience and were overwhelmed by the bureaucratic requirements. Children's research activity has since risen substantially leading to the appointment of a part-time research nurse and a clinical trials assistant, development of a dedicated research clinic, inclusion of research in junior doctor induction and teaching sessions, and planning a parent’s research group. Catherine says “it does seem to have changed the way the whole department views research; studies are now seen as an opportunity and a routine part of clinical work rather than an unnecessary burden”. The paediatric team won the hospital "Researcher of the Year Award" in 2012 for embedding research in the Department. Catherine’s case illustrates the breadth of benefits that accrue from previous research experience, and the support of a go-ahead Research & Development Director.
A sensible strategy

Embedding the nation’s research strategy in the NHS makes very good sense. However we find continued dissociation between the clear aim to increase research to benefit patients as articulated by the National Institute of Health Research and successive UK Governments, and the poor integration of research and health care delivered through the NHS.

A clinical and regulatory ethos is needed in which opportunity for every child admitted to NHS care to enter into a relevant clinical research study is considered an index of quality care. This is essential to bring about a reduction in uncertainties in care more speedily and at lesser expense than conventional clinical trials.

The use of clinical data to support NHS evaluations, such as audits, surveys, reviews, bench-marking, and outcomes analyses, as well as research, must be strengthened. A similar vision has been articulated by the UK Department of Health (The power of information: Putting all of us in control of the health and care information we need; 2012). Life-long follow-up can in theory be achieved through better use of linked NHS electronic clinical records. Duplication of processes common to NHS care and research, such as

INCREASING RESEARCH CAPACITY THROUGH USE OF NIHR CRN RESOURCES

Alder Hey Children’s NHS Foundation Trust is a research active organisation leading original research and supporting national studies in the NIHR Clinical Research Network portfolio. It has associated income available for consultant research programmed activities (PAs) through annual CLRN allocation, Research Capability Funding (previously Flexibility and Sustainability Funding), NIHR programme and grant “directly allocated costs” and commercial income.

Within the Trust, sub-speciality teams are generally small with 4-5 consultant posts. There is limited scope to incorporate additional PAs into individual or team job plans. Paediatric rheumatology is a very research-active group generating original research awards, contributing to NIHR CRN portfolio recruitment and attracting commercial income. In 2011, the Trust Research Business Unit identified the equivalent of eight PAs of research income (CLRN, FSF and commercial) directly attributable to Paediatric rheumatology research activity. This was mobilised in late 2011 to create a new consultant post in paediatric rheumatology comprising six Direct Clinical Care PAs and two Supporting PAs. The net effect has been to redistribute ‘research PAs’ among the entire paediatric rheumatology team providing identified and protected research time within each individual’s job plan and a significant increase in research capacity. Funding for the new consultant post has been underwritten by the Research Business Unit for five years, by which time it is anticipated that growth in research income will be sufficient for long term sustainability. The post holder commenced in March 2012.
follow-up evaluations, should be eliminated as this is wasteful of time and resources, and burdensome to patients.

There is growing evidence that patient outcomes are better in research-active settings. The involvement of clinical teams working collaboratively with research leaders is crucial to increasing and strengthening child health research and the NHS consultant workforce will always outnumber consultant level paediatricians employed by Universities, the NIHR and Research Councils. We have identified outstanding examples of NHS consultant paediatricians taking up the opportunities presented by the new NHS research structures to become research leaders.

Regrettably despite the clearly stated desire to increase research activity in the NHS the pressures for the majority of consultants are mainly in the opposite direction through increasing restriction of contracted time to 10 Programmed Activities. At present the winning of funding for Consultant Programmed Activities for research is seen as the only route through which NHS Consultants are able to engage in clinical research activity. This is an inadequate response to maximise the potential of children’s research to improve patient care and service delivery. Employers should set out the allocation in time for contributions to research in consultant job plans with achievements against predefined goals assessed at annual appraisal. For those consultants that have demonstrated ability and inclination to increase research activity, progression should not be limited by pressures from NHS Trusts. They should be supported to maintain Continuing Professional Development to carry out high quality audits, surveys and systematic reviews, health service evaluations and quality improvement projects, and supervise trainees undertaking research.

**Recommendations**

- We urge the National Commissioning Board and Clinical Commissioning Groups to include the development of clinical trials integrated into clinical care in commissioning frameworks

- Opportunity for entry into publicly funded, investigator led clinical trials designed to resolve uncertainties in treatments already in wide use should be considered an index of quality, and a standard of care

- Publicly funded, investigator led clinical trials designed to resolve uncertainties in treatments already in wide use should be subject to regulation that is proportionate, and not the same as that necessary for trials of novel experimental agents

- We recommend a review of processes that are common to the NHS and research, and consideration of ways in which duplication can be avoided, quality improved, and costs reduced

- We recommend the establishment through NHS commissioning structures, of a national network of infant and children’s follow-up centres staffed by personnel trained to deliver neurodevelopmental and other assessments to a standard required for clinical care and for research outcome evaluation, and to ensure this information may be shared appropriately; this will reduce the burden imposed upon families, and the costs of clinical care and research
- We recommend placing responsibility for capture of clinical data used for NHS purposes and research with clinicians, ensuring that high performing teams and NHS Trusts are recognised and rewarded in a manner analogous to the recognition received for recruitment to NIHR portfolio studies.

- We recommend NHS employers adopt a flexible and individual approach to increase the contribution of paediatric consultants to children’s research at a level commensurate with ability and career aims.

- We recommend the RCPCH fulfils a pivotal role through the professional expectation that every consultant paediatrician should be supportive of research.

- We recommend that core research competencies are considered an essential criterion for appointment to a substantive NHS paediatric consultant post, reflecting the current trainee curriculum.

- We recommend the RCPCH Education Division maintains a commitment to promoting access to Continuing Professional Development activities in generic research competencies.

- We recommend NHS employers support access by paediatric consultants to Continuing Professional Development in relevant research skills and knowledge.
6 University employed paediatricians

“The Wellcome Trust does not receive a high number of grant proposals from paediatricians .... it is a specialist area in which we receive few proposals”
Dr Pamela Reid, Grants Management Department, The Wellcome Trust

6.1 Clinical academics

Clinical academics are doctors who treat patients, but are also responsible for educating medical students and for carrying out research. A clinical academic spends about half his or her time as a practising doctor and half in teaching and/or research. Clinical academics are employed by universities; their research can cover a range of aspects of health and disease as well as health services research. The substantial expansion in NHS consultant numbers has not been matched among the academic workforce, resulting in a fall in the proportion of the latter in relation to the total consultant workforce from 8.7% in 2001 to 5.2% in 2011 (table 6.1).

Table 6.1 Paediatric academic and NHS consultant numbers, 2001 to 2011

<table>
<thead>
<tr>
<th></th>
<th>2001</th>
<th>2003</th>
<th>2005</th>
<th>2007</th>
<th>2009</th>
<th>2011</th>
</tr>
</thead>
<tbody>
<tr>
<td>Professors</td>
<td>82</td>
<td>75</td>
<td>84</td>
<td>79</td>
<td>87</td>
<td>88</td>
</tr>
<tr>
<td>Reader/Senior Lecturers</td>
<td>109</td>
<td>107</td>
<td>95</td>
<td>85</td>
<td>89</td>
<td>100</td>
</tr>
<tr>
<td>Total Consultant Level Academics</td>
<td>191</td>
<td>182</td>
<td>179</td>
<td>164</td>
<td>176</td>
<td>188</td>
</tr>
<tr>
<td>Total NHS Consultants</td>
<td>1995</td>
<td>2337</td>
<td>2544</td>
<td>2761</td>
<td>3081</td>
<td>3400</td>
</tr>
<tr>
<td>Academic Consultants as % of total NHS Consultants</td>
<td>9.6%</td>
<td>7.8%</td>
<td>7.0%</td>
<td>5.9%</td>
<td>5.7%</td>
<td>5.5%</td>
</tr>
</tbody>
</table>

6.2 Professors of Paediatrics

The number of Professors of Paediatrics has remained static for almost a decade, with 80 Full Time Equivalents at Professorial level identified in 2004 and 83 in 2010 (Medical Schools Council 2011) (figure 6.1); 44 of the 83 were aged 56 or above. Of 31 medical schools, 4 had no Professor of Paediatrics and 20 had 3 or less, among which were all medical schools in Northern Ireland, Wales, and Scotland (Fig 6.2). A census carried out by the RCPCH two years later in 2012 identified 88 consultant level paediatricians with a primary professorial appointment. The RCPCH 2012 census also indicate that no university has more than three Professors of Paediatrics in the same speciality other than Imperial and Oxford each of which has four Professors of Paediatric Infectious Diseases. The balance of clinical academic
paediatricians at professorial and senior lecturer/reader levels is best in infectious diseases, allergy and respiratory medicine at Imperial, Oxford, Brighton, St George’s University of London, and Southampton. In contrast across all paediatric specialities many professors and senior lecturers/readers are single handed with no colleague at professorial, or senior lecturer/reader level. This mismatch is particularly striking in neonatal medicine. In this speciality the RCPCH 2012 census identified twelve professors and fourteen senior lecturers/readers but in only four universities are there appointees at professorial as well as senior lecturer/reader level.

Figure 6.1  Paediatric clinical academic staff by grade, 2004 to 2010 (Medical Schools Council 2011)

Figure 6.2  Professors of Paediatrics by Medical Schools, 2010 (Medical Schools Council 2011)
6.3 Senior Lecturers/Readers

The Higher Education Funding Council for England has funded “New Blood” Clinical Senior Lectureships in partnership with NHS trusts and Universities. Each award is made for five years and the fifth and final round of scheme has now been completed. Paediatrics did well, securing 19 of the 188 posts (10%) (table 6.2). The career progression of these appointees will be a matter of considerable interest. This notwithstanding the Medical Schools Council head count of Senior Lecturers/Readers in Paediatrics shows a fall from 127 in 2004 to 115 in 2010 of whom 77 were aged above 45. The RCPCH 2012 census identified 100 Senior Lecturers/Readers in Paediatrics.

Table 6.2 Higher Education Funding Council for England “New Blood” Clinical Senior Lectureships awarded 2006/7 to 2010/11

<table>
<thead>
<tr>
<th></th>
<th>2006/7</th>
<th>2007/8</th>
<th>2008/9</th>
<th>2009/10</th>
<th>2010/11</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total</td>
<td>38</td>
<td>38</td>
<td>40</td>
<td>37</td>
<td>35</td>
<td>188</td>
</tr>
<tr>
<td>Paediatrics</td>
<td>6</td>
<td>3</td>
<td>4</td>
<td>3</td>
<td>3</td>
<td>19</td>
</tr>
<tr>
<td>Percentage awarded to Paediatrics</td>
<td>15.8%</td>
<td>7.9%</td>
<td>10.0%</td>
<td>8.1%</td>
<td>8.6%</td>
<td>10.1%</td>
</tr>
</tbody>
</table>

RCPCH 2012
6.4 Lecturers

Only 32 (20 men and 12 women) Paediatric Clinical Lectureships were identified in the Medical Schools Council Survey conducted in 2010, a fall from 43 in 2004 (25 men and 18 women), and the lowest number to-date (figure 6.1). The NIHR lectureship programme was established to support senior clinical trainees who have completed a doctorate or equivalent, and show “outstanding potential for continuing a career in academic medicine”. NIHR Clinical Lecturers spend 50% of their time in specialist clinical training and 50% in research or education training. Paediatricians secured 22 of 578 (3.8%) awards made in last six years, including 2 of 114 appointments in the 2011/2012 round (table 6.3). In Scotland lecturers spend 80% of time in clinical care and 20% in research (personal communication, Dr Mandy Drake); this may not provide sufficient time for competitive research.

Table 6.3 NIHR Clinical Lectureships awarded 2006/7 to 2011/12 NIHR Clinical Lectureships, total new posts and posts in paediatrics (Data courtesy of NIHR Trainees Coordinating Centre)

<table>
<thead>
<tr>
<th></th>
<th>2006/7</th>
<th>2007/8</th>
<th>2008/9</th>
<th>2009/10</th>
<th>2010/11</th>
<th>2011/12</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Paediatric ACL posts</td>
<td>0</td>
<td>4</td>
<td>4</td>
<td>7</td>
<td>5</td>
<td>2</td>
<td>22</td>
</tr>
<tr>
<td>Total ACL Posts</td>
<td>80</td>
<td>86</td>
<td>95</td>
<td>99</td>
<td>104</td>
<td>114</td>
<td>578</td>
</tr>
<tr>
<td>Percentage awarded to Paediatrics</td>
<td>0%</td>
<td>4.7%</td>
<td>4.2%</td>
<td>7.1%</td>
<td>4.8%</td>
<td>1.8%</td>
<td>3.8%</td>
</tr>
</tbody>
</table>

6.5 Comparison with other specialities

Only two specialties have achieved an increase in the number of academic clinical FTE (lecturer, senior lecturer, reader, professor) between 2000 and 2010, general practice (153 to 184, an increase of 20%) and general medicine (973 to 1282, an increase of 32%). In general medicine this represents a rise in the number of professors from 465 to 580, senior lecturers/readers from 515 to 526, and Lecturers from 140 to 236. In contrast paediatrics has seen an overall decline by 10.2% in the total academic workforce, from 246 to 221 FTE. In adult medicine the ratio of professors to senior Lecturers/Readers is 1.1:1, but in paediatrics the ratio is reversed with a ratio of 1:0.7; further two-thirds of senior lecturers/readers are over the age of 45.

6.6 National Institute of Health Research Faculty

The NIHR Faculty consists of staff funded by the NIHR who lead or support research, or evaluate the effectiveness of healthcare interventions and policies. There are four categories of faculty membership, senior investigators, investigators, associates and trainees. The NIHR has also established “Research Professorships” to lead the translation of research from “campus to clinic” and from “bench to
bedside”. NIHR Professorships are directed at researchers at consultant grade who have “an outstanding record of clinical and applied health research and its effective translation for improved health” and who are at an early stage of their careers. Of the eight NIHR professorships awarded in the first round in 2012, none was in a paediatric speciality; of over 200 NIHR senior investigators, 8 are paediatricians.

6.7 Paediatricians on Research Boards and Councils

Only six of around 500 Medical Research Council and Wellcome Trust Board and Committee members is a paediatrician but we do not know whether or not this is the consequence of paediatricians failing to put themselves forward. The National Research Ethics Advisors Panel provides strategic leadership on ethical issues in relation to research and associated matters pertaining to service development, policy, training, quality assurance and stakeholder engagement; of its 13 original members not one was a paediatrician; a paediatrician is among the new membership announced in October 2012. The NIHR Strategy and Advisory Boards number some 50 individuals; not one is a paediatrician nor is there a paediatrician on the UKCRC Board which is made up of senior representatives of all partner organisations.

6.8 Gender distribution of clinical academics

The proportion of women is rising in paediatrics (Figure 6.3) as in other specialities, resulting in further extension of the time to appointment to a tenured academic position. Many European countries provide flexible parental leave of up to a year that may be taken up by either father or mother, and so allow families to choose how best to support their children and their careers; the UK has recently adopted a similar approach though this is as yet little known and child care responsibilities continue to be placed disproportionately on women, with men perhaps not realising that they are able to opt for flexible working while their children are young.

Table 6.4 Women as a percentage of total number of Professors, Senior Lecturers/Readers and Clinical Lecturers in Paediatrics, Adult Medicine, and General Practice (Medical Schools Council 2011)

<table>
<thead>
<tr>
<th>% Women</th>
<th>Paediatrics</th>
<th>Adult Medicine</th>
<th>General Practice</th>
<th>Surgery</th>
</tr>
</thead>
<tbody>
<tr>
<td>Professors</td>
<td>14.5%</td>
<td>12.0%</td>
<td>20.4%</td>
<td>6.1%</td>
</tr>
<tr>
<td>Senior Lecturers/Readers</td>
<td>29.5%</td>
<td>25.2%</td>
<td>37.4%</td>
<td>9.4%</td>
</tr>
<tr>
<td>Clinical Lecturers</td>
<td>41.1%</td>
<td>36.4%</td>
<td>53.2%</td>
<td>11.1%</td>
</tr>
</tbody>
</table>

6.9 Grant funding

The RCPCH consultant survey carried out in 2012 identified 22 consultant level paediatricians in receipt of a grant as principle/lead investigator from the Wellcome Trust, 34 from the Medical Research Council, 52 from the National Institute of Health Research, 107 from a national charity and 123 from a local charity. The largest numbers of grants received by paediatricians are from local charities.
Towards a solution

The paediatric specialty of Community Child Health includes services delivered outside hospital settings; research may be epidemiological, public health, clinical or health services, and relate to long-term and disabling conditions, vulnerable families, child maltreatment and mental health. There are important interfaces with social care, education, the criminal justice system as well as other sectors within the health service. Research capacity in community child health is particularly underdeveloped in the UK for reasons that are complex and multifactorial. The Community Paediatric Research Group has recently discussed how research could be promoted despite these difficulties. The Group has outlined a strategy to address key unanswered questions using “safeguarding” as an exemplar. The Group proposes a national collaboration of NHS and university employed community child health paediatricians and other professional groups, that would deliver a research programme commencing with a systematic review of existing evidence, followed by the development of a series of research studies with delivery through a research network supported by the British Association for Community Child Health working closely with affiliated groups in public health, mental health, disability, audiology and child protection.

Summary and recommendations

The reduction in paediatric clinical lecturers and the aging senior paediatric academic workforce is a major concern. There have been previous warnings of the adverse impact of a small and diminishing number of paediatricians in academic positions, exacerbated by the loss of visibility brought about by the disestablishment of Departments of Paediatrics and Child Health during university restructurings (Anonymous 1997; Levene & Olver 2005). The Research Assessment Exercises of the past decades, and the coming Research Evaluation Framework, do not encourage universities and medical schools to act with anything other than extreme caution, so that the appointment of young academic paediatricians with potential, but limited track records, is all but precluded, further adding to poor growth in academic numbers and increasing the difficulties for those remaining. The likelihood is that without action, this situation will result in further erosion of the science base for child health over the next decade.

We show in Section 4 that paediatric trainees have done well in securing around one in ten of all Academic Clinical Fellowships awarded since the commencement of the Integrated Academic Training Programme in 2006, with more than half continuing in research training. It takes 10-15 years from entry into the Integrated Academic Training Programme to reach readiness for appointment to a senior lectureship. The experience gained by these young people is likely to be beneficial regardless of their final career choices, but if they are to become children’s research leaders, they will need the mentorship of a senior academic over this period, opportunity to work with stimulating research groups and availability of lecturer and senior lecturer posts at the right time and in the right location. Yet as we have seen, children’s research is short on critical mass, and appointment opportunities to research-active positions, whether primarily NHS or University based, are too often complex and inflexible.
At present the requirements of academic life, juggling research, professional, teaching and clinical responsibilities, while supervising postgraduate students, championing translation research, and attending to substantial administrative responsibilities, are all too commonly falling upon lone senior lecturers and professors with inevitable detriment to their research and scholarship, thus perpetuating a downward spiral for children's research. For example the small number of paediatricians in receipt of an award from the Wellcome Trust and MRC is in accord with the views expressed by the heads of these institutions that they receive few grant applications from paediatricians. A tenfold higher number of paediatricians report an award from a charity; the largest numbers of grants received by paediatricians are from local charities. Many such awards do not fulfil the criteria necessary to be included in the NIHR research portfolio, chief of which are that they should be awarded in open competition and should have received independent peer review. The consequence is that these studies are not eligible for support through a research network and paediatricians do not improve their ability to develop rigorous studies able to hold their own in open competition.

An expansion in child health research capacity requires partnerships of paediatricians, other clinicians and non-clinical scientists working in concert across institutions supported by professional organisation; such innovative solutions to diminished capacity are particularly needed for “hard to reach” groups of children (box).

**Recommendations**

- We regard as crucial, the need for an acute expansion in clinical and non-clinical post-doctoral positions, lectureships and senior lectureships in child health, linked to established professorial-led research groups

- To further address the problem of insufficient critical mass we consider it essential to develop cross-institutional multidisciplinary research programmes spanning the breadth of children’s research

- We recommend alignment of children’s charities with Research Councils, industry, the NIHR and universities to create new positions, and support collaborative child health research groups
7 Young people, parents and the public

“We need to encourage patients to be more demanding of their doctors and NHS institutions when it comes to offering the chance to take part in research” Dr Jonathan Sheffield, CEO of the National Institute for Health Research Clinical Research Network

7.1 User involvement is an important component of the research process. In paediatrics, this spans the involvement of children, young people, and their parents and carers. User input encompasses identification of need, prioritisation, study design, dissemination of outputs, and enhancing public awareness of research (box).

“In recent years we have worked with a group of families who help us to decide our research priorities, to design and conduct the research studies and then help to drive dissemination of the evidence. Families’ priorities and views often differ from those of clinicians and academics so it has taken a big adjustment from researchers but it has transformed the enjoyment we get out of doing research and the effectiveness of our research effort.”

Professor Stuart Logan, Director of PenCRU, Exeter University

“Having been a midwife, prior to having my little girl, I have had a keen interest in research. I found that having a child with special needs often led to me being treated as if I too had special needs which I found incredibly frustrating. When I found out about PenCru I jumped at the chance to help with their research in any way that I could. So far this has meant that I have joined their family faculty, have joined in with them to further their research into how communication with nursing staff can be improved for children with difficulty communicating while in hospital and try to encourage other parents to join the family faculty. I finally feel that I am able to contribute to both a better future for my little girl and I am able to use my knowledge and research skills. It is rewarding to have my intelligence acknowledged and my opinion valued.”

Amy Francis

7.2 There has been considerable attempt in the UK to advance this agenda. The organisation INVOLVE was established in 1996 to support active public involvement in NHS, public health and social care research and is now funded by the National Institute of Health Research. It is one of the few Government funded programmes of its kind in the world. The INVOLVE Research Project database hold details of published and unpublished projects in health and social care that include the active involvement of members of the public; 31 of 236 projects currently listed include children’s issues (accessed 22/08/2012). INVOLVE has been among the foremost in supporting the active involvement of children in research and providing supporting guidance (Kirkby 2004).

7.3 The Medicines for Children Research Network encourages children and families to work closely with researchers and supports this in a number of ways such as through a Young Persons Advisory Group, involvement of parents in Clinical Study Groups and Local Research Networks, and public engagement activities.

7.4 The James Lind Alliance receives a major part of its funding from the National Institute of Health Research. This brings patients, carers and clinicians together to
identify and prioritise uncertainties and unanswered question about medical treatments. For example one priority setting partnership aims to address uncertainties relevant to preterm birth.

7.5 Sense about Science (http://www.senseaboutscience.org) is a charitable trust that aims to help people to make sense of scientific and medical claims about research and understand the underpinning evidence. The Charity provides advice and information, delivers programmes, and leads campaigns.

7.6 Children, as much as other patients, deserve a fair deal. This means that where there are treatment uncertainties, they must be offered opportunity to participate in a high quality, appropriately regulated, clinical trial, so that they have equal chance of receiving a beneficial treatment, and not receiving a useless or harmful treatment. However, it is not uncommon for parents, and staff, to have important misunderstandings about research and specific studies. Some researchers report attempts to manipulate the situation, for example, agreeing to randomisation but then withdrawing if their child is not randomised to their preferred arm (almost invariably the new intervention). This reflects poor appreciation that a new treatment may not be efficacious. Public awareness of the nature of clinical research is not strong and partners need to work together to improve this situation. Participation in other forms of research is also needed to advance understanding of heath and disease (box).

**Eleanor and Edward Smith’s parents’ account**

“**Having two children with cystic fibrosis; a genetic condition that is life shortening, progressive and all encompassing, the importance of clinic research cannot be expressed strongly enough. We are a family that like solutions, but cystic fibrosis has no cure, so doing anything that can help with the treatment and give our children and others like them some hope for the future, is very important to us. For this reason as parents we were very keen to take part in any research that will further the understanding of CF, its effects and treatments. By taking part in research we feel less helpless and more hopeful, and it has given our children a sense of pride in their contribution to the future.”**

**Words from Eleanor...**

“My name is Eleanor Smith and I am part of the cystic fibrosis run-in study. As a person with cystic fibrosis I feel that it is very important to help find a cure. When I participate I perform a variety of simple tasks, designed to have a close look at how a cystic fibrosis body works. Everyone is very polite and nice to me, and although I have needle phobia I still feel in a comfortable environment to do the blood test required. I enjoy going, and feel proud and excited to be part of it.”

**Henry Bartlam**

As an infant, Henry had very severe asthma and recurrent pneumonia. As a toddler he had a deformed chest due to lung over-inflation and dependent on treatment with oral steroids. A letter was published in the Lancet on one severe asthmatic child in Israel who had been given nebulised budesonide with clear benefits. Henry’s paediatrician, Professor John Warner, contacted the author and
between them they arranged for the pharmaceutical company to send a consignment of the medication. Henry had a spectacular response which led Professor Warner to conduct a placebo controlled clinical trial on steroid dependent asthmatic pre-school children. This trial showed benefit and led to the medication being approved for use.

“Henry has remained well with a progressively diminishing requirement for treatment and is a successful and very active young man.” Professor John Warner

Words from Henry...

“I was obviously too young to fully appreciate the severity of my asthma and in fact I have very few negative memories of the illness. That said, it was very much central to my growing up, whether it was from the routine of using the nebuliser twice daily or the regular check-ups with doctors and Professor Warner. But I can honestly say that as I have grown older, the impact that the illness has had on my day-to-day life has been increasingly small - I’ve always led an active life and rarely has asthma got in the way. Obviously much of this comes down to the impact and success of the nebulised budesonide had on my health. I have no doubt (and my Mum would be the first to vouch for this) that I would not be able to paint such a positive picture if Professor Warner hadn’t taken the decision to try it out all those years ago.” Henry Bartlam

7.8 There have been several studies seeking to investigate the views of parents, and recruitment to research studies from their perspective (Mason et al 2000; Freer et al 2009; Shilling et al 2011). These have shown that although medical and nursing staff often hold back from discussing research from concern that they are overburdening parents, parents themselves do not mind being approached even in the most difficult situations. Parents also value verbal discussion with a clinician with whom they are familiar over and above written information. There is good evidence for the areas that families consider important when considering research; these include explanation of the regulatory approvals and safety monitoring, concise written information backed up by on-to-one discussion with member of the clinical team, and clear understanding of the practicalities of participation. The RCPCH supports the recommendation for a Children’s Charter proposed by the Children’s Outcomes Forum, and will collaborate with key partners, including parents, children and young people to ensure it reflects the importance of research.

7.9 Children’s research has specific issues where guidance is required to supplement the legal position. For example the Scottish Children’s Research Network with the Central Legal Office of NHS Scotland has set out the principles of “assent” and “consent” that are considered best practice, over and above any legal requirement.

7.10 The RCPCH aims to expand the number of lay publications and web content produced in parallel with professional materials, building on the precedent established by the RCPCH Medicines Committee that has producing leaflets aimed at parents and children that explain the use of medicines.
Summary and recommendations

The public often seem uncertain about types of research, how research is regulated, and the implications of biomedical research, which are often not well-translated by the media.

Patient engagement has grown substantially in recent years but user-leadership in child health research remains limited.

"I've seen many families who like us, have endured the unimaginable pain of seeing their child suffer and in some cases die from cancer. My daughter received the most amazing treatment and had the best possible outcome. That's why I'm so keen to promote further research so fewer families have to suffer." Neil Ranasinghe

Since 2006, when inspired by his daughter's successful treatment, Neil has been involved in writing and reviewing a lot of documentation for parents of children with cancer. He is on a number of committees and working groups and provides editorial support with guidelines, funding applications and patient information sheets. Neil has also formed PORT: Paediatric Oncology Research Team along with other parents whose children have been involved in clinical trials for cancer. One of their core aims is to work with medical professionals to write and review information for parents and children relating to clinical trials.

All areas of children's research need support, not only those where the aim is disease cure, but also where the aim is best management of chronic incurable conditions (box).

“My son, 9, had a hemispherectomy aged 18 months. He doesn’t speak, understand speech or use signs or pictures. He doesn’t know he is disabled; so as long as his needs are met he is a loud, cheerful, cheeky boy with a sweet nature. My experience as a parent leads me to believe that the most difficult but most important question is how to involve children who have very limited or no communication abilities in how they feel about their health and their experience of medical intervention.”

Antonia East

Recommendations

- Researchers and research regulators must heed messages from parents, young people and children about the way in which they wish to be involved in and be told about research

- The RCPCH will collaborate with key partners to ensure the Children’s Charter proposed by the Children’s Outcomes Forum reflects the importance of research. The RCPCH communications team has an important role to play in improving public understanding of children’s research through the media, and print and web materials aimed at families and the public, produced in parallel with professional materials

- We recommend the RCPCH Parents and Carers Group works closely with parents, young people and children, health care professionals, and other organisations active in this area, to advance active advocacy for research to benefit child health and wellbeing
8 Translating research into policy and clinical practice

“...policy makers have to work hard to identify problems, to specify research that might help solve them, and to receive and use the results of research” Kogan and Henkel, Analysis of the Rothschild Report 1983, page 9

8.1 Child wellbeing in the UK

Children are a nation’s future but the UK does not fare well in assessments that have been made of child wellbeing. UNICEF conducted a comprehensive assessment of the lives and well-being of children and young people in 21 countries in the industrialized world (Unicef 2007). Objective indicators in 6 dimensions were considered (material well-being, health and safety, education, peer and family relationships, behaviours and risks, and young people’s own subjective sense of well-being). The Netherlands heads the table of overall child wellbeing, ranking in the top 10 for all 6 dimensions; the UK and the United States are in the bottom third of the rankings for 5 of the 6 dimensions (Table 8.1). Of note is that no relationship was identified between child wellbeing and the wealth of a country.

Table 8.1

The chart below presents the findings of this Report Card in summary form. Countries are listed in order of their average rank for the six dimensions of child well-being that have been assessed. A light blue background indicates a place in the top third of the table; mid-blue denotes the middle third and dark blue the bottom third.

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<th>Dimension 3</th>
<th>Dimension 4</th>
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<th>Dimension 6</th>
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<td>Educational well-being</td>
<td>Family and peer relationships</td>
<td>Behaviours and risks</td>
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</table>

OECD countries with insufficient data to be included in the overview: Australia, Iceland, Japan, Luxembourg, Mexico, New Zealand, the Slovak Republic, South Korea, Turkey.
The indicators for the domain “Health and Safety” were the number of infants dying before age one year per 1,000 births, the percentage of births that are low birth weight (<2500g), percentage of children age 12 to 23 months immunized against measles, DPT, and polio, and deaths from accidents and injuries per 100,000 children up to the age of 19. For all indicators excepting the last (where it ranks second to Sweden), the UK ranks in the lowest third.

This report was followed in 2011 by further research commissioned by UNICEF UK that concluded that the pressures imposed by materialism and inequalities, compounded by declining family life were prominent in the UK (Child well-being in the UK, Spain and Sweden: The role of inequality and materialism Ipsos MORO Social Research Institute in Partnership with Dr Agnes Nairn, 2011).

The UK has the highest child mortality rate in Europe; child deaths in the UK from meningococcal disease, pneumonia and asthma exceed that of the Netherlands, Germany, France, Sweden and Italy and it has been estimated that the UK has 1500 excess all cause child deaths per annum in comparison to Sweden (Wolfe 2011). Whether these disparities can be explained by differences in national policies is uncertain as there has been little in the way of comprehensive assessment of comparative children’s healthcare across Europe. It is noteworthy that in the UK, first healthcare contact is usually delivered by General Practitioners, of whom only a third has received any training in paediatrics (Wolfe 2011).

8.2 Recognition of the need for children’s research

The UK Department of Health National Service Framework for Children, Young People and Maternity Services encapsulates eleven standards. Although the scant evidence base for child health care is acknowledged, for example section 9.16 of the standard “The Mental Health and Psychological Well-being of Children and Young People” states “The requirement to ensure an evidence-based approach to practice presents a particular challenge to professionals working in Child and Adolescent Mental Health Services” and “….. services have to rely frequently on either extrapolating research findings from abroad or from adult literature”, there is no mention of the importance of promoting and supporting clinical research to advance the evidence-base. In the final paragraph of the “Core Standards” (Core Document: National Service Framework for Children, Young People and Maternity Services, Department of Health 2004) the need for policy research is acknowledged but no mention is made of the importance of experimental medicine, clinical trials, and biotechnology development aimed at the needs of children.

8.3 Translating research into national policy

There are good examples of successful translation of research into policy, such as the substantial literature on air pollution and children’s health that provided support for the introduction of the London low emission zone and the “Back to Sleep” programme (Box).

Many areas affecting child health today remain in need of specific policy development and advocacy. These include the effects of passive cigarette smoking, air pollution, speeding limits and daylight saving time as a means to reduce accidents. One in three UK children is now overweight or obese, yet a cornerstone of UK Government strategy is “responsibility deals” with food and drink manufacturers despite the lack of evidence of any positive impact; the emphasis is
on identifying overweight through monitoring; the need for research to identify causes, implement preventive policies and identify interventions that work is not mentioned.

**From evidence to policy, implementation and improved outcomes: the Sudden Infant Death Syndrome story (Wolfe 2012)**

Prone sleeping position was noted as a possible risk for Sudden Infant Death Syndrome in 1944 (Abramson 1944) and by the 1970s there was reliable evidence. This was not accepted widely until the 1980s. (Gilbert, Salanti et al. 2005) nor did systematic preventive efforts begin until the early 1990s, and then only in a few countries where this was followed by a precipitate decline in SIDS (Fig 8.1).

**Figure 8.1 SIDS rates (per 1000 live births) in 4 Countries of Western Europe**

In the preceding years there had been a rise in SIDS deaths, coinciding with a fashion for promoting prone sleeping by child-care experts that had begun in the 1940s (Gilbert, Salanti et al. 2005). This advice was indirectly supported by research and advances in neonatal intensive care in the 1960s. (McKee, Fulop et al 1996) Front sleeping position continued to be advised in child-care manuals until the late 1980s (Gilbert, Salanti et al. 2005). It took many years to understand the risks of prone sleeping but even once the evidence was quite clear, there was further delay in translating this knowledge into public health advice, and then into a change in baby sleeping position. The lag between gaining understanding of risks and devising policy to mitigate those risks provides useful lessons for child health policy. Furthermore the processes of policy-making and organization of public health services affects the success or failure of policies. The policy responses of 4 countries illustrate these points. The Netherlands and the UK adopted national, and France and Germany, regional campaigns. The decline in SIDS happened earlier in the Netherlands and the UK than in France and Germany the curve representing the rate of the former lies to the left of the latter on the graph (Fig). The Netherlands was the first country to see a change in infant sleeping position practice. This followed the presentation of
research results demonstrating an association between sleep position and SIDS at a public lecture in 1987. However, it was widespread media interest, rather than a public health campaign that appeared to be responsible for the change in parenting practice. Before 1987, 46% of babies slept face down; this figure dropped to 19% afterwards (McKee, Fulop et al. 1996). Sudden infant deaths fell from 1.04 to 0.44 per 1000 live births in the subsequent five years (de Jonge, Burgmeijer et al. 1993).

In the UK, a small study demonstrating a convincing rise in risk of SIDS associated with prone sleeping (Fleming, Gilbert et al. 1990) led first to a local, then a national campaign led by a charity, the Foundation for the Study of Infant Deaths (FSID), that distributed information and public health advice to health professionals throughout the country. National media interest was fuelled when a television presenter’s baby died of SIDS and she generously allowed her family’s experience to further the FSID campaign. These efforts prompted the Government to issue a policy statement but although this was circulated to health professionals it was not widely implemented until after 1991 following a national campaign “Reduce the Risk” bringing together the efforts of FSID and central Government. The numbers of babies dying from SIDS dropped by half within a year (Foundation for the Study of Infant Deaths 2009).

Centralised national public health organizations with effective means of disseminating information, helped to implement the public information campaign in the UK. In contrast, in France, a non-Governmental organization handled the SIDS-prevention campaign in regions. A planned national information campaign was delayed by elections that diverted political attention until 1995. Germany also embarked on efforts to prevent SIDS through non-Governmental organization led distribution of information leaflets to two regions. Once again, it was national media attention on the regional campaigns that is likely to have helped spread the message more widely (McKee, Fulop et al. 1996). The lack of a strong centralized public health function and relatively autonomous regions in both countries may have hindered the national dissemination and implementation.

8.4 Policy development

The RCPCH has a strong leadership role to play in influencing national policies that affect child health, notwithstanding the wider social, economic, and ideological determinants that must be considered, and that evidence alone may be insufficient to effect change. Decisions concerning the specific issues to be targeted require recognition that the landscape may change rapidly, and that influencing policy may require a nimble response. Conversely a long view is required in some areas. There must be consideration of what is desirable, what is achievable, and whether the RCPCH role is to be a broker of evidence and knowledge, that is providing relevant information and evidence to policy-makers, the media and the public, an advocate for specific policies, or an organisation that seeks to shape or influence particular policies. The current capacity of the RCPCH to extend its influence upon national policy and clinical guidelines is a key consideration; for example the American Academy of Pediatrics health policy materials include a mix of position statements, technical reports, reviews, and parent leaflets. The RCPCH Clinical Standards section has a potentially crucial role in conducting high quality systematic reviews and meta-analyses. This requires extending in-house expertise or partnering with academic institutions. Greater activity by the RCPCH will require investment, in staff with strengthened and widened skill sets, enhanced electronic technologies
that facilitate rapid efficient dialogue with paediatricians, and flexible financial reserves to support new work.

Policymaking should be underpinned by evidence, but where there are gaps in evidence judgement is required to balance the pros and cons of options and researchers should be supported to target efforts to meet needs. The translation of evidence to effective policy may also be improved by commissioning high quality evidence syntheses, a priori consideration of conflicting recommendations, incorporation of planned evaluations of effect and impact, and pilot studies prior to national implementation. Barriers to be broken down include mistrust between researchers and policy-makers, the political naivety of the former, and the scientific naivety of the latter. Paediatricians and researchers can be supported in developing skills in communicating with policy-makers. Institutional and organisational arrangements can help bridge the gap between researchers and policymakers in several ways such as policy dialogues, policy briefs that are topical, timely, of high scientific quality, informed by discussions with parents and carers, children and young people, and produced in succinct, structured format. The mass media and civil society groups can further support the policymaking process. There is, as yet, no organisation tasked with strengthening the evidence base for child health policy.

8.5 Translation of research into clinical practice

The UK has been at the forefront of attempts to place evidence centre stage of the NHS care. The National Institute of Clinical Excellence (NICE) was established in 1999. A key aim was to reduce variations in NHS care around the country, the so called ‘postcode lottery’. By carefully evaluating available evidence, particularly about new and expensive medicines, it was felt that clinical decision making would be assisted, and the introduction of poorly evidenced treatments, often as a result of media and public pressure, would be avoided. NICE has been an arm’s length body funded by the UK Department of Health. Following the passage of the Health and Social Care Bill in 2012, NICE will become a non-departmental public body with an expanded remit to develop quality standards for social care.

NICE outputs include evidence-based guidelines, public health guidance on best approaches to healthy living, wellbeing and disease prevention. Technology appraisals are recommendations on the use of medicines, medical devices, diagnostic techniques, surgical procedures and health promotion activities. Between 2007 and 2011 NICE produced a total of 472 outputs; of these 35 were wholly specific to paediatrics (7.4%); in a further 53 (11.2%), primarily directed at adult healthcare, some paediatric material was included (Table).

The scant research evidence base for children’s health care is impeding the development of effective guidance. The development of technology appraisals in children has been hindered by the principle adopted by NICE of not developing these if the medicines in question are not licensed, or used off label. This is a particular problem in paediatrics. Many widely used children’s medicines, and indeed over 90% of neonatal medicines, are unlicensed or used off-label and are likely to remain so in the absence of evidence to support their use. Supporting evidence is only one of nine criteria used by NICE for topic prioritization and does not therefore stand alone in determining whether guidance generation will proceed. In paediatrics other criteria, such as overall burden in relation to total population, also results in lack of prioritization.
Consensus methodologies play an increasing role in these situations; NICE guidance on child maltreatment, published in 2009, is one example. However the harm that can be done through formalising a non-evidence based approach must not be underestimated. “Expert opinion” perpetuated prone sleeping for decades (Box). Boosting the research base will mean health policy is not as reliant on expert opinion or based upon associations rather than tests of causality and this will reduce risk since consensus views often merely reflect the prevalent view of the moment. If a consensus view is wrong, all patients will continue to receive the harmful treatment. In the absence of evidence it is questionable that “expert” opinion based guidelines are really better than having no formal guidance; in the former case all patients will receive either the right or the wrong approach, but in the latter at least some patients will always be spared harm. A more subtle and possibly even more adverse impact of consensus based approaches is that health professionals, particularly the younger, are dissuaded from challenging current dogma.

Translation of guidance into improved patient outcomes requires a host of new Quality Improvement tools, ranging from measurement of the problem through systematic surveillance, audit of practice against existing standards, in depth methodologically rigorous case reviews to drill down and establish the root causes of errors, publication of performance in quality accounts and a culture of national learning. It is essential that these NHS processes are conducted to a high standard.

Table 8.2 NICE Outputs 2007-2011 (Data gathered by Rita Ranmal and Rosalie Lear)

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## Summary and recommendations

### The need for primary research

The paucity of NICE outputs directed at children by the limits imposed upon guideline generation from lack of primary evidence is damaging to children’s healthcare and wellbeing. The dangers of basing clinical guidance on consensus or expert opinion have been highlighted. The need for research extends through many domains; for example health economic modelling techniques require to be developed that provide an objective metric of the value to the nation of children’s research where numbers are small but impact large because of the benefits to life-long health. The relevance and importance of children's research does not feature prominently in national policy. This contrasts with high level statements acknowledging the importance of child wellbeing. This dissociation between recognised need (the latter) and paucity of evidence to effect change (the former) requires recognition at the highest level. The dangers of basing policy on associations must also be recognised. High quality research designed to test causality is the gold-standard approach that should underpin policy development no less than clinical practice. Hence children’s policy research and evaluation must be promoted.

### The role of the RCPCH in contributing to the national evidence base

The RCPCH is achieving greater effectiveness and efficiency in contributing to the child health evidence base by integrating its activities, improving two-way communication through the development in 2012 of an electronic portal to all UK paediatricians and becoming a cardinal national repository of up-to-date information on children’s health. The RCPCH aims to facilitate the surveillance of rare and common conditions in children, and
integrate this with other activities that are also require close communication with consultant level paediatricians throughout the UK. This will include the development of registers and rapid surveys. We intend to use robust methodologies, state-of-the-art technologies, external independent peer review, and engage with industry, and with the new UK structures, including Public Health England and the National Commissioning Board. We are delivering a growing portfolio of national audits (www.rcpch.ac.uk/nationalaudits) and other national programmes such as the Clinical Outcome Review Programme, Child Health Reviews-UK (www.rcpch.ac.uk/chr-uk) that combines epidemiological evaluation of child deaths with in-depth case assessments, and developing care pathways (www.rcpch.ac.uk/allergypathways). The RCPCH has also been at the forefront of greater transparency, being among the first organisations to attribute the outcomes of audits to named providers and place these in the public domain, and promote the involvement of parents and young people across all activities. We intend that these activities continue to grow.

**Partnerships and coalitions**

The SIDS case history indicates that there are several elements of a successful public health policy campaign. These include reliable synthesis of primary research evidence, a clear message, focused independent sector leadership, strategic partnerships, national dissemination ideally through a centralized public health system, clear lines of communication with Government, and generation of strong media interest.

**Recommendations**

- An increase in the generation of primary research evidence is needed across multiple domains, including health technology assessments, method development studies, economic evaluations and health services research, as well as efficacy end effectiveness research, to strengthen the generation of high quality evidence-based guidance for children by the UK National Institute of Clinical Excellence

- We recommend the Department of Health, Department of Education, NICE and Commissioning Boards establish formal co-ordinated processes for highlighting evidence gaps in clinical guidelines and in national policies that affect child wellbeing

- The RCPCH is committed to growing its contribution to the national evidence base for children’s policy

- We recommend the development of a small number of “Policy Road Maps” to direct priority areas agreed by RCPCH Council, which encompass synthesis of research evidence, identification of target audiences and strategic partners, a communications and media strategy, and involvement of young people, families, non-clinical scientists and public figures.

- We recommend that national health policies that affect children should be informed by primary research evidence and subjected to rigorous evaluation

- We recommend the establishment of strategic alliances between organisations that have a major focus on child health and wellbeing, to influence national policy
9 Increasing and strengthening children’s research: the way forward

“Mankind owes to the child the best it has to give” Geneva Declaration of the Rights of the Child, 1924

9.1 Time for change

The science of children's healthcare, paediatrics, is relatively new. The first children's hospitals were founded less than 150 years ago and many paediatric subspecialities have only achieved recognition in the last two to three decades. The Royal College of Paediatrics & Child Health was founded in 1996, after a tumultuous break-away of the British Paediatric Association from the Royal College of Physicians. Children's research brings added dimensions, that of parental involvement, small size, and rapidly changing physiology. In some respects it is therefore not surprising that children's research has yet to reach maturity. International recognition of this problem has led to initiatives such as StaR, Standards for Research in Child Health, an international group of researchers, regulators, and editors, who aim to improve children's research design, conduct and reporting (Hartling et al 2012).

In the UK we have identified positive developments supportive of child health research, principally the creation of the Comprehensive Research and topic specific networks including the Medicines for Children Research Network, described in section 2, the introduction of the Integrated Academic Training Programme, described in section 4, and the strong commitment of successive UK Governments to strengthening biomedical research.

However there is dissociation between the desire to increase research, and the activities of the NHS, universities and research funders. The recognition of poor coordination between these bodies led to the establishment of the Office for Strategic Co-ordination of Health Research (OSCHR) in 2007, but there is no evidence of continuing activity by OSCHR and the problem remains unresolved.

We identify other dissociations, those between the clearly articulated recognition of the importance of children's health and wellbeing, the steps that must be taken to identify and implement effective means of improving this, and the wider implications of child wellbeing for population health; in other words there is inadequate recognition of the relevance of children's research, not only to their health and wellbeing, but also to that of the nation, and successive generations.

We draw attention to the necessity for children to be included in research endeavour, and for children's research to regain lost ground. Children, and the adults they will become, deserve a UK wide culture that includes them in scientific enquiry, scholarship and innovation, and recognises the possibilities to improve population health through research focussed upon the early years that extends from basic science, clinical studies, through to improved delivery of health care and translation of research evidence into national policy.

Research addressing the causes and prevention of diseases that have their origins in early life has potential to turn the seemingly inexorable tide of growing
population burden arising, for example, from obesity and cardiovascular disease. The science underpinning efforts in tissue regeneration, stem cell biology and neural plasticity - areas that offer most hope for health in old age - require basic research that begins with an understanding of these processes in infancy. If the potential of early year’s research is to be harnessed, a long vision, and a resourced strategy is required, that has as its metric, the health of the nation.

9.2 The environment, infrastructure and capacity for children’s research in the UK

The disparity between evidence gaps for children’s health care and research effort is marked. This is reflective of a tension that remains, despite deliberations over a century, played out in the Haldane Report (1918), Rothschild Report (1971), House of Lords report “Priorities in Medical Research” (1988), Culyer Report (1994), and Cooksey Report (2006), of the balance that must be struck between research commissioned to address knowledge gaps of importance to health, and a strategy that encourages scientists to follow their own ideas. Both the “bench to bedside” model is insufficient; so too overly directed, “commissioned” research. Great advances have followed on from percipient clinical observation, scientific curiosity, and serendipity. This notwithstanding, mechanisms to drive research endeavour to particular areas of need are also needed.

Clinicians and non-clinical scientists working in child health must be a part of multidisciplinary research teams if the unprecedented opportunities provided by the wealth of post-genomic technologies are to be seized. However capacity in the UK for research involving infants, children and young people is small. Children’s research groups lack critical mass, and are spread thinly over many institutions. The number of children’s research leaders is small. The university appointed paediatric workforce has been in decline for over a decade; universities and medical schools have recognised neither obligation nor responsibility to support child health research. The Integrated Academic Training Programme has attracted high calibre paediatricians with a majority going on in continued research training. However it will take a decade for this young cadre of future research leaders to complete their training, a period that will see further shrinking of the already diminished number of research active paediatricians. In the short-term this crisis is best addressed by supporting strong, cross-institutional partnerships that bring together clinical and non-clinical scientists, paediatricians, and non-paediatricians, in research programmes addressing the science of children’s disease, and health and wellbeing across the life-course. There is also an acute need to increase the number of posts for young researchers, and grow the research leaders of the future. These requirements call for innovative funding models and collaborative effort.

9.3 The NHS

The NHS offers opportunity that is internationally unparalleled to advance healthcare through research. The establishment of the research networks has been welcomed but the failure to grasp the full potential of the NHS, and achieve a truly supportive arena for children’s research is a major frustration. The turmoil inflicted upon the NHS by successive reorganisations, the European Working Time Directive, the European Union Clinical Trials Directive, and responsibilities that are becoming increasingly devolved, are conditions that are leading to an environment within the NHS that is all too often disengaged from research at best and destructively dismissive of its importance at worse.
Closer integration of research and clinical requirements that would hasten a reduction in uncertainties in care, reduce costs, and the burden imposed upon families, and achieve greater efficiencies, are within reach. For example there is all too often an unnecessary duplication of registers and databases of clinical information; shared resources would be time and cost-efficient. Research regulation must be mindful of the needs of infants, children, young people, and their families. Opportunity for entry into publicly funded, investigator led clinical trials designed to resolve uncertainties in treatments already in wide use should be considered an index of quality, and a standard of care, and not subject to the same regulatory burden as trials of experimental agents, but to a proportionate approvals process. This approach is exemplified by the children’s cancer networks in which trial entry is considered de facto best practice, an approach that has led to rapid acceleration in survival (Stock et al 2008).

Research that involves children often requires long-term follow-up assessments to evaluate clinically relevant outcomes. Regular assessments of children’s health and wellbeing are a component of UK Government policy (Healthy Child Programme, UK Department of Health 2009). Yet every researcher planning a study that requires follow-up evaluations is required to address the problem anew. A network of nationally commissioned centres staffed by child health professionals with requisite expertise in neurodevelopmental and other health assessments, could serve the needs of good clinical care and researchers alike.

Many long-term outcomes can in theory be obtained from linked NHS records, as can data for clinical trials, disease registers, and a range of NHS evaluations crucial to improved care, such as audits, outcomes assessments, and dashboards. The quality of much NHS data is poor. Giving clinicians responsibility for data capture, and incentivising this in similar manner to that in which recruitment into NIHR portfolio studies is recognised, would lead to rapid improvements in both quality and completeness (Spencer & Modi 2012).

All NHS paediatric consultants will be users of research, many will contribute, and some will be research leaders. Certainly the NHS consultant paediatrician has more to offer than recruiting to clinical trials, which has been a large component of endeavours to increase their involvement in children’s research. NHS consultant paediatricians outnumber academic consultants in a ratio that exceeds 15:1; they are closest to children and their families, knowledgeable about the practicalities of clinical research, and best placed to inspire trainees, characterise disease phenotypes and ask relevant research questions. A keen awareness of research, encompassing good understanding of the UK regulatory framework, familiarity with basic methods including health services research techniques, and critical appraisal skills, must become integral to the make-up of all NHS paediatricians. We recommend the appraisal of paediatricians should include the extent of their contribution to reducing uncertainties in the care they provide, and evaluating outcomes. This requires that the NHS allocates time in paediatric consultant job plans for research activities at a level commensurate with career goals, and related continuing professional development.
9.4 Maintaining and monitoring progress: the RCPCH commitment

The obstacles to progress we have identified require leadership and decisive action if they are to be overcome. A long vision and a consistent and sustained focus is necessary if the potential of early life, children’s and young people’s research is to be realised. The Royal College of Paediatrics and Child Health is committed to doing what it can to help bring the wide variety of organisations and individuals together to help create this vision. We are strongly supportive of widening the involvement of NHS clinicians in research, monitoring trends, assessing impact, and strengthening child health policy development. We will lead the way in establishing a Science Advisory Board to direct these activities.

Clinicians are crucial to successful research endeavour that involves children. We identify as a major impediment to progress the inadequacies in opportunity for exposure to research, and the acquisition of core research skills and knowledge of research regulation and governance, that are essential for all paediatric trainees, not only those in academic career pathways. Without such exposure, the science of medicine is diminished, and the ability of clinicians to fulfil a cardinal obligation, reducing uncertainty in patient care, is weakened. The RCPCH is committed to fostering a strong ethos, with research regarded as an index of high quality care, improving the acquisition of core, generic research skills and their appraisal for all paediatric trainees, developing flexible approaches to obtain research experience, and providing advice and mentorship for trainees considering a research-active career. Objective criteria for research skills and experience for all paediatricians have been defined and are included in the research competencies section of the paediatric e-portfolio. A Guide to Training in Child Health Research has been prepared and is widely available, and an Academic Training Committee established to supervise activities.

Parents and young people have a vital role to play in advocating for research to improve the delivery of care, reduce treatment uncertainties, ensure that research evidence is incorporated into national clinical guidelines and policies, and that these are implemented, audited, and evaluated. The MCRN has led the way in effecting closer engagement with young people. The challenge now is to improve the involvement and understanding of parents of the benefits of children’s interests being better represented in basic science, clinical, health services and policy research, ensure that the public, and the wider academic communities, including ethicists and lawyers, are well informed about the need for children’s research, and the underpinning processes and regulations, and grow advocacy for children’s research that is driven by parents and young people. We will therefore be supporting the development of the Children’s Charter proposed by the Children’s Outcomes Forum and will aim to ensure it reflects the importance of research. In particular we will do what we can to ensure it sets out the expectations of children, young people and their families in relation to research to understand the biology of their health and disease, reduce uncertainties in the treatments they receive and improve health care delivery.

There is no over-arching focus for children’ research in the UK. The economic environment is challenging and only one national children’s research charity has a research spend that exceeds £1.5 million per annum, largely precluding their ability to support large clinical trials and major research programmes, establish substantive research posts, create regular opportunities for research experience, or
fund infrastructure. The creation of more effective translation of evidence into national policies that improve child health, and active advocacy to sharpen the focus on children’s research and maintain momentum, would be best served through collaboration between the many organisations concerned with improving child health.

We believe that these goals can best be met by the establishment of a new partnership organisation, the remit of which would be to co-ordinate efforts, focussing on areas that cannot be accomplished readily by a single body. We propose a forum that enables charities to retain their unique identities while collaborating strategically with larger organisations, public funders and industry, to increase and strengthen children’s biomedical and health services research and the translation of research evidence into national policies, standards and guidelines to improve life-long health. We have therefore begun consultations with children’s research charities, and other organisations, on the establishment of a UK “Children’s Research Collaboration”.

We call also upon the Royal Colleges, parents, young people and the public, the NHS, universities, industry, philanthropists, charities and research councils and the UK Government to consider the recommendations in this report, and join us in tackling the challenges identified in order to benefit children, their life-long health, the health of the nation, and successive generations.
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Rowe, Dr Ian
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Tuffrey, Dr Catherine
Turnberg, Professor Lord Leslie
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Voight, Dr Jana
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Wardle, Dr Stephen
Warren, Dr Janet
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