

## Guest Editorial

### Improving child health using evidence, when little exists

In most resource-rich countries, children are generally healthy. In Europe and the US, only about 12% of children have what is considered a chronic condition;<sup>[1]</sup> and of these, most — like mild intermittent asthma — are associated with low morbidity, and pose little threat to mortality. Beyond the first year of life, hospitalisation or death among children is rare; and when death occurs, unintentional injury is the most common cause.<sup>[2]</sup> While childhood antecedents of adult diseases exist, quantifying such relationships — and the effect of interventions to disrupt them — requires long-term follow-up. Over the decades of follow-up, morbidities and interventions change. Because of the low prevalence of serious paediatric illness, outcomes that are difficult to measure, and conditions that take years to manifest, it has been difficult to build an evidence base for child-health practice analogous to that available for adults.

Although new and creative techniques are being developed to prove and improve the practice of medicine,<sup>[3]</sup> the highest level of evidence to assess the effects of treatments remains the randomised controlled trial, either as a sole generalisable study, or as aggregations of data in the form of systematic reviews or meta-analyses. A soon to be published analysis in which we were involved found RCTs and systematic reviews to be more common among studies of treatments involving adults than among those involving children;<sup>[4]</sup> specifically, RCTs were over three times more common, and systematic reviews nearly six times more common.<sup>[4]</sup> Whether this gap in evidence quality is an appropriate reflection of the different morbidities between adult and child health, or whether it represents a failure of the child-health profession and those involved in publishing medical data, remains a matter of debate. In either case, the challenges to building an evidence base are different for children with and without chronic health conditions; and, for the purpose of commenting on evidence-based child-health practice, these two populations should be considered separately.

For the almost 90% of children who are healthy, the goal of health care is to keep them that way. This is generally accomplished through screening (e.g. for iron deficiency anaemia), counselling (e.g. on the use of bicycle helmets), and prophylaxis (e.g. immunisation). Outside of immunisations, however, reviews of commonly recommended paediatric practices consistently demonstrate little or no high-quality evidence to support them.<sup>[5]</sup> Of the 12 distinct paediatric conditions reviewed by the United States Preventive Services Task Force as potentially important aspects of general paediatric preventive care, screening, or counselling, recommendations were inconclusive for six of them (screening for neonatal hearing loss, development dysplasia of the hip, serum lead levels, serum lipid profiles, obesity, and speech/language delay). Of the five positive recommendations from the Task Force, three are simple screening blood tests (for sickle cell disease, PKU, and hypothyroidism), which are often performed outside the purview of the healthcare provider.<sup>[6]</sup> Specific challenges to establishing an evidence base pertaining to healthy children, therefore, include difficulty parsing out risk groups from among the general child population (i.e. a screening practice may be effective among certain children but not others), the inherent variability with which counselling is performed, and difficulty proving the effectiveness of long-term prevention practices. Furthermore, many of the conditions for which we wish to provide counselling or prophylaxis need public-health or community-wide solutions that are more likely to be beneficial than clinic-based interventions that occur in varying community contexts, which

may not be comparable with one another. This last point is reflected in a recent UNICEF report which stated that only 16 of 40 indicators of child wellbeing in resource-rich countries had anything to do with the provision of healthcare.[7]

For the just over 10% of children with chronic health problems, the challenges are fundamentally different. Children with cystic fibrosis, cancer, or sickle cell disease, for example, have common measurable outcomes, in the same way as adults with heart disease and diabetes mellitus and — more importantly — different from otherwise healthy children. However, there are simply not enough children with chronic conditions in any one centre or geographic region from which to extract robust or generalisable study results. The challenge, therefore, becomes conducting expensive and logistically difficult multi-centre trials. In the US, the Children's Oncology Group, which enrolls children from across the country in collaborative clinical trials, is one example of how this challenge has been met. In the UK, Medicines for Children Research Network has been established to serve a similar purpose. While the multi-centre trials required by these models tend to yield more generalisable results than single-centre studies, such studies have unique potential threats to internal validity that involve the effects of each individual centre on study results.[8]

Building the evidence base for child health is possible. For most children who are healthy, this is going to involve an appreciation that the majority of threats to child wellbeing — poverty, violence, and low educational achievement — lie outside traditional medicine. For children with major chronic disease, such as cystic fibrosis, inflammatory bowel disease, or type 1 diabetes mellitus, harnessing the remarkable advances in research will require coordinated multi-centre studies that involve long-term follow-up.

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## References

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