Outcome and input

Editorial

The demand for evidence-based medicine has encouraged the study of outcomes in children who have neurodevelopmental disabilities. However, the approach to these issues, often based on standard procedures that relate to diseases in adults, seems naive. The nature of neurodisability makes the study of outcome very difficult. And before we can judge outcome, we need to look more closely at input into the studies. (Note a curious semantic issue here – the opposite of outcome is not income but input. However, 'income' may have an effect on outcome.)

One has to consider the country, culture, and ethnicity of the children studied. Rates of cerebral palsy (CP) are perhaps surprisingly consistent across the 'developed' world[1], although regional variations are apparent. But should we assume that the population of children with, for example, athetosis or autism in Western Australia is the same as that in Scandinavia or California. Probably not. The very small babies who contribute to the population with disabilities in developed countries are unlikely to survive in less developed parts of the world. Babies who 20 years ago would have developed the traditional spastic diplegia of 'prematurity' are probably less commonly seen now, but they may form the bulk of the population with CP in 'developing' countries. It is not always easy to be certain of the diagnosis of CP, it is even more difficult to get strict diagnostic criteria for conditions like attention-deficit–hyperactivity disorder and autistic spectrum disorders. The importance of getting the diagnosis right is emphasised[2].

The next issue is who comprises the study population: the whole population of children with the condition within a circumscribed area and population base? Or does the population consist of referred children and, if so, by whom: a doctor or a physiotherapist? Is the referral determined by the parents of the child? A new treatment may attract the attention of families, particularly if it is on the Internet; parents may struggle to get their children to the centre where the promised effective intervention is available. Such a biased sample will clearly affect the outcome.

Of the clinical population, who will be studied? Will the 'intervener' look at all children with the particular condition? Or will there be some selection? For example, if a child is deemed unsuitable for a procedure, will s/he be excluded from a study which will possibly use an intervention such as Botox? And what criteria will be applied to the study population? Will the population be restricted by sex and/or age? Will it be divided into age groups such as 0 to 5, 5 to 10, and 10 to 15 year olds. Or will the whole population be considered, assuming a person's signs and symptoms at 5 years are the same or similar to those at 15 years (which is unlikely). Will all of the diagnosed children be treated or will treatment be restricted? For example, will all the children with the symptomatology of autism be treated or only those with a non-specific autism, and will those who have CP or tuberous sclerosis in addition to autism be excluded? This raises the familiar question in neurodisability of whether similar signs and symptomatologies arise from the same cause.

Children of different ages may or may not have had earlier interventions, ranging from orthopaedic surgery to psychodynamic therapy. How will these early interventions affect the outcome? These different treatment paradigms may be very important. Both age and previous treatment experiences affect how an individual may respond to the suggestion of new interventions. As one parent of an individual with severe disabilities said to me during a home visit, 'We've had doctors. There's nothing you can do', even though I was pretty certain that in the 15 years since the initial diagnosis the condition had changed and we could have helped. Equally, the young person or child may feel very cynical about the intervention and may not cooperate throughout. This leads onto the families' perception of the treatment. If families have referred their children to a new and supposedly effective intervention, they are likely to be more positive about the outcome than those who have been randomly selected and persuaded to participate in the new treatment studies.

Finally, we need to assess whether the child is facing other complications such as physical health problems common to the whole childhood population, for example, asthma; starting a new school; or becoming aware of disturbing family social situations where perhaps relationships are breaking down. Now it is time to start looking at specific outcomes. The lines for this are set out by Butler[3].

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References

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