Evidence based medicine

Editorial

The two important articles on melatonin in this issue make a very interesting contrast.[1,2] In the first we requested and got a personal view based on years of experience and the available evidence, laced with clinical nuances and subtleties, to guide those of us who are less familiar with the field. The other, by a team who are also very experienced in the use of melatonin, has followed an evidence based approach and shows how few hard data are available when rigid criteria are applied. Surprisingly, a proposed multicentre trial in the UK has not so far been funded, despite scientific validity and ethical approval (Appleton RE, personal communication). It does not seem quite attractive enough to the unelected judges on the various charities' governing bodies. It is a very apposite example of the difficulties in proving that what we do is effective, even when most of us believe it to be so.

Increasingly our pay masters, more so for colleagues in North America than elsewhere, are demanding high levels of proof before funding an intervention. However, even in common disorders there are embarrassingly few data. Examples include the rather scanty high grade evidence for clinical differences between drug therapies for the epilepsies, summarized in a recent report,[3] or for various therapy modalities in cerebral palsy.[4,5] This leads to continuing controversies, especially in expensive areas where claim and counter claim give an impression of muddle and confusion to families desperately wanting treatment for currently incurable conditions.

Lack of funding creates a catch-22 situation, but this is not the only issue. For example, in Duchenne muscular dystrophy the use of corticosteroids is becoming widespread, but we do not have long term population based trials to demonstrate the balance of benefits versus side effects such as weight gain, growth restriction, cataracts, and vertebral fractures. It is now unlikely ever to be possible to set up a long term randomized placebo-controlled study because what parent would want to risk their child being in the placebo arm? In a field such as ours, where many conditions are individually rare, it can be extremely difficult to gather data in sufficient numbers. In the relatively common problem of acute brain trauma most studies are underpowered.[6] Investigators are researching important topics which deserve publication and discussion, but to address this problem many studies would have to be on a national rather than regional or local level. To emulate the success of paediatric oncology, where in many countries most children are in some kind of study even if simply an observational one, requires an infrastructure as well as willpower. If one can imagine having to fill in a complex form about each patient we see after each consultation so that a central registry could monitor their progress, one can also imagine the enormous resource implications.

It becomes even harder when trying to prove the value of a non-drug intervention, as for example the effectiveness of specialist staff in hospital clinics. The latter include specialist nurses in epilepsy, who can advise and educate parents on management, or in neurodisability, who can help children with cerebral palsy in the home, for example in using gastrostomies, or refilling baclofen pumps. Not all have to be nurses: in the United Kingdom non-nursing specialists for people with neuromuscular conditions have until recently been funded by a major charity. They helped families come to terms with the diagnosis and in later stages to cope with issues such as the bureaucratic maze of wheelchair provision or home adaptation. In practical terms they provide as much value as any medical input. However, proving this to service providers is a very different matter.

The limitations of a purely evidence based approach has been wittily lampooned in an article on the lack of Grade 1 evidence that parachutes are worth wearing if jumping from aeroplanes.[7] However, the extensive correspondence that followed pointed out its benefits too, for example, in showing unexpected disadvantages of seemingly obvious treatments. Evidence based medicine must be adequately funded so that new therapies can be made available to people who will benefit.

Returning to melatonin, it has undoubtedly been helpful in some, but not all, of my patients. However, it is not licensed for use in many countries, even though in the United States preparations are widely available as a food supplement. The different preparations available also need to be compared. In view of the undoubted benefits shown in both the articles in this journal, and the importance of sleep disorders for stressed families coping with children with other difficulties, we must prove that treatments like this are worthwhile. We can then apply them with the skill learnt from our expert colleagues.

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