

TESTING TREATMENTS

Chapter 5, 5.1

5 Dealing with uncertainty about the effects of treatments

In this chapter we look at the uncertainties that almost invariably surround the claimed effects of treatments, whether new or old. For example, few would probably question the routine use of oxygen in people who have had a heart attack, yet there is no good evidence that it helps, and some evidence that it may cause harm. This uncertainty has never been adequately addressed¹ and many other effects of treatments are disputed.

DRAMATIC TREATMENT EFFECTS: RARE AND READILY RECOGNIZABLE

Only rarely will the evidence be so clear-cut that there is no room for doubt about whether a treatment works.² In such cases the treatment effect is often dramatic and immediate. Take the heart rhythm disorder known as ventricular fibrillation, where muscle contraction in the ventricles (lower chambers) of the heart becomes wildly uncoordinated. This is a medical emergency – death can occur in minutes. The technique of ‘zapping’ the heart with a direct electrical current from a defibrillator applied to the chest is used to restore the heart’s normal rhythm; when successful, the effect is virtually instantaneous.

Other dramatic effects (see also Chapter 6, p70) include drainage of pus to relieve pain from abscesses, blood transfusion for shock caused by severe haemorrhage, and insulin (a hormone produced by the pancreas) for diabetes. Up to the 1920s, patients

with diabetes had short lives and suffered immensely, wasting away with uncontrollably high blood sugar levels. Very quickly, the initial results of animal tests led to the use of insulin in patients, with outstanding success – their response was near miraculous at the time. Another example from that era was the use of liver – later shown to be a source of vitamin B12 – for patients with pernicious anaemia. In this then fatal type of anaemia, the numbers of red blood cells gradually fall to disastrously low levels, leaving patients with a ghostly pallor and profound weakness. When these patients were given liver extract they recovered rapidly, and vitamin B12 is now prescribed routinely for this form of anaemia.

Some examples from the beginning of this century highlight similarly dramatic results.

Laser treatment of portwine stains

The birthmarks known as portwine stains are caused by permanent and malformed dilated blood vessels in the skin. Commonly occurring on the face, they persist and often darken as the child matures, and can be seriously disfiguring. Numerous treatments were tried over the years including freezing, surgery, and radiation, but with little impact and many side-effects. The introduction of laser treatment brought impressive results: improvement is usually seen after a single laser session in most types of lesions, and the damage caused by dispersion of heat from the laser to the surrounding skin tissues is temporary.^{2,3}

Imatinib for chronic myeloid leukaemia

Impressive results have also been seen in patients given imatinib for chronic myeloid leukaemia.^{4,5}

Before imatinib was introduced in the late 1990s, this type of leukaemia responded very poorly to standard treatments. When the new drug was tried, initially in patients who had not responded to standard therapy, the outlook for patients improved greatly. Imatinib stabilizes the disease, appears to prolong life substantially by comparison with the pre-imatinib era, and has mostly mild side-effects. It is now regarded as the first treatment option.