Efficacy and safety of medicines

Introduction

Medicines need to balance efficacy and safety.

A medicine is beneficial when it produces the desired effect (efficacy) with an acceptable level of side effects (safety).

Efficacy vs. effectiveness

Efficacy relates to how well a treatment works in clinical trials or laboratory studies. Effectiveness, on the other hand, relates to how well a treatment works in practice after a medicine is generally available.

Benefit vs. risk

The benefit of taking a medicine must exceed the risks of taking it.

Even though no technology is completely safe, the perception of acceptable risk level may vary between patients, healthcare professionals and regulatory authorities.

Experience has shown that some patients are more willing to accept higher risks than regulators and healthcare professionals.

Efficacy and safety monitoring

After a medicine becomes available to patients, the efficacy and safety still need to be monitored. This is called ‘pharmacovigilance’.

The World Health Organisation (WHO) defines pharmacovigilance as '[…] the science and activities relating to the detection, assessment, understanding and prevention of adverse effects or any other drug-related problem'.

Today, before a medicine is made available to patients, it has typically been tested (depending on the disease) on at least 5.000 persons for a limited time period.

The long-term efficacy and safety of the medicine must be monitored and continuously evaluated while on the market, or – in other words – under 'real-life' conditions.

As side effects are reported, more information will become available on the safety of a medicine.

If a patient experiences any side effects, it is recommended that they talk to a healthcare professional. This includes any possible side effects not listed in the package leaflet. In many EU member states, side effects can be reported directly via the national reporting system available on the websites of the National Competent Authority (NCA).

Further Resources

- World Health Organization (2002). *The importance of pharmacovigilance: Safety monitoring of medicinal products*. Geneva: World Health Organization. Retrieved 23 June, 2015, from http://apps.who.int/medicinedocs/pdf/s4893e/s4893e.pdf?ua=1.
- World Health Organization (2004). *Pharmacovigilance: Ensuring the safe use of medicines*. Geneva: World Health Organization. Retrieved 23 June, 2015, from http://apps.who.int/medicinedocs/en/d/Js6164e/ (Retrieved 23 June, 2015).

Attachments

- Presentation: Efficacy and Safety of Medicines

  Size: 373,525 bytes, Format: .pptx

  A presentation describing the efficacy and safety of medicines, which can be adapted for own use.

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