Good Off-Label Use Practice

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WHY IS THIS IMPORTANT FOR RARE DISEASES?

Off-label use of medicines is the practice of using a medicine outside of its authorized indications. In rare diseases, the use of medicinal products off-label is widespread and primarily driven by the lack of authorized medicines for specific indications.

GOLUP is an initiative that aims to provide a clear framework on when and how the off-label use of medicinal products can safely take place. A clear approach on the issue is becoming increasingly urgent due to the current trend of promoting the prescription of medicines off-label for reasons other than the medical need of patients. In fact, some European Union Member States are imposing prescribing guidelines that effectively promote off-label use for the sole purpose of reducing healthcare spending.

The below criteria, drawn together by independent experts, stem from decades of research and clinical practice and are applicable to the rare diseases field as well as to other areas where off-label use frequently occurs, including paediatric care.

The identified criteria do not aim to limit off-label use, but seek to promote a harmonized approach for its occurrence in order to maintain the highest levels of patient safety and minimize adverse events.

Off-label use of medicinal products should only occur if all the following criteria are met:

1. Presence of a medical therapeutic need based on a current examination of the patient by a suitably qualified health care professional.
2. Absence of authorised treatment and licensed alternatives tolerated by the patient or repeated treatment failure.
3. A documented review and critical appraisal of available scientific evidence favours off-label use to respond to the unmet medical need of the individual patient.
4. Patients (or their legal representative) must be given sufficient information about the medicines that are prescribed to allow them to make an informed decision.
5. Presence of established reporting routes for outcomes and adverse events linked to off-label use.

The Declaration for Good Off-Label Use Practice is supported by a coalition of European organisations that are dedicated to ensuring that high standards of patient care are upheld and that progress in medical research and innovation is achieved.

EFNA · EBC European Brain Council · emsp European Multiple Sclerosis Platform · EMA European Medical Association · European Alliance for Access to Safe Medicines · EARLS

EPDA European Parkinson's Disease Association · ATM Associazione Italiana Miastenia e Malattie Immunodegenerative Amici del Besta Onlus · EuMGA European Myasthenia Gravis Association · Encephalitis Society The brain inflammation charity · ANMAR Associazione Nazionale Malati Reumatici Onlus · EMEA

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