Adverse Events and unlicensed/off-label use of medicines
A Consensus Statement

We believe there is an urgent need to address patient safety issues around the unlicensed/off-label use of medicines.

As a minimum criterion, we believe that unlicensed/off-label medicines should only be used in cases where there is no licensed product available that meets the medical need. If this condition is satisfied, a number of points still need to be met to ensure patient safety is not compromised.

Adverse Events (AE) reporting is a critical component in the continuous monitoring and assessment of medicines safety. However there is currently no mandatory mechanism in place for recording and/or reporting AEs in the use of unlicensed/off-label medicines. In addition, the lack of awareness of AE reporting amongst the general public is alarmingly low.

We believe action is required to:

- **Establish the number of AEs** relating to unlicensed/off-label use of medicines
- **Introduce a professional Code of Practice for mandatory reporting** by healthcare professionals of AEs involving unlicensed/off-label medicines as currently there is no mechanism for recording or reporting AEs in this context
- **Improve public awareness** so that patients are aware of when unlicensed/off-label medicines are used and the importance of AE reporting, and that they give their consent for those medicines to be used

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Background

When a pharmaceutical company seeks a licence to market a medicine it must demonstrate that the product is safe, effective and of good quality through laboratory testing and clinical trials. A licensed medicine is granted a Marketing Authorisation (MA) and the clinical indications, dosage, age of patient, method of administration, precautions and other information from the MA are presented in the Summary of Product Characteristics (SPC).

An **unlicensed medicine** is a drug that does not have a marketing authorisation. **Off-label** refers to the use of a drug which has a marketing authorisation but is used for a condition, at a dose, via a route or for an age that is not listed in the SPC for that drug.

An **Adverse Event (AE)** is a reaction to a drug that is not expected or wanted. It is important for side effects to be reported to enable the regulatory authorities to make medicines safer for everyone. Reporting AEs makes it possible for the regulators to find possible previously unidentified hazards and other new information on the side effects of medicines. The results from AE reports are used to monitor the safety of a medicine and evaluate the risk-benefit balance of the medicine.

As medicines used unlicensed/off-label have not gone through the strict licensing regulations, the need to monitor and report AEs in these medicines is even more vital.