

WATAG Advisory Note

Rule of Rescue - Medications - Guidance for Health Service Providers

The “rule of rescue” refers to an exceptional situation within health services, where an indication is not listed on the Statewide Medicines Formulary and approval might not otherwise be granted. As a point of reference WATAG has adapted the Pharmaceutical Benefits Advisory Committee criteria as a guide to decision making in WA public hospitals and health services.

Four factors should be considered in exceptional circumstances to guide decisions when considering an individual patient request in these situations. When all four of the following factors apply concurrently, the criteria for ‘rule of rescue’ are satisfied.

- There are no alternative treatments available in WA for patients with the specific medical condition for which approval is being sought. This means that there are no non-pharmacological or pharmacological interventions for these patients.
- The medical condition for which approval is requested is clearly defined and is severe, progressive and expected to lead to premature death, severe morbidity, loss of function/productivity and/or quality of life. The more severe the condition, or the younger the age at which a person with the condition might die, or the closer a person with the condition is to death, the more influential the rule of rescue might be in considering individual patient approval.
- The medical condition defined in the request applies to only a very small number of patients. Again, the fewer the patients, the more influential the rule of rescue might be in considering hospital approval. However, hospitals should also be mindful that they cannot cater for every circumstance.
- The proposed medication provides a worthwhile clinical improvement sufficient to qualify as a rescue from the medical condition. The greater the rescue, the more influential the rule of rescue might be in the considering hospital approval.

Other relevant factors may also need to be considered. Overall, the rule of rescue should supplement, rather than substitute for, evidence-based considerations. A consideration of whether the rule of rescue is relevant is only necessary if the hospital Drugs and Therapeutics Committee (DTC) would be otherwise inclined to reject an application to use the medication. If a hospital DTC concludes that the rule of rescue is pertinent, it would then consider whether the application is sufficiently influential in favour of approving exceptional use.

WATAG recommends that use of medicines under the rule of rescue be managed by hospitals under a process of individual patient approval (IPA). That is, an application should be made to the hospital DTC on an appropriate form with supporting information. Mechanisms for expediting an IPA should also be developed and approved by the DTC to facilitate rapid decision-making in urgent situations. Additional approval by senior hospital executive or business manager should be considered where there are substantial cost and budget implications. A reasonable estimate of the expected costs to the hospital should be provided, taking into consideration the duration of treatment and the potential number of patients to be treated. Once approved, and in the interest of equity, any subsequent patient presenting under similar circumstances should be eligible for

treatment of the specified condition under the rule of rescue, subject to an IPA submission in each case. However, by definition, the number of eligible patients should be very low.

Hospital staff are reminded that medicines used under the rule of rescue are usually for rare clinical conditions and are therefore unlikely to be registered by the TGA. That is, usage would be off-label and the quality and safety of the treatment may not be well established. It is important that informed consent be obtained from the patient (or guardian) and that the risks and benefits associated with the novel therapy are explained. It is also important to maintain good records of medications approved under the rule of rescue (as with all IPAs) including a record of drug usage and clinical outcomes. Information about IPA medication usage and outcomes should be available (in de-identified format) to inform clinical practice within the hospital and at other health services in WA and Australia.

Chairman, WATAG