

# Sample Expanded Access Policy

## ACCESS TO INVESTIGATIONAL DRUGS POLICY

### 1. PURPOSE

This document describes COMPANY NAME's position, and reflects our Corporate Policy on how patients in medical need may be able to access our new medicines before they are fully approved for use on prescription.

### 2. SCOPE

We cover two areas in this document:

1. Where patients participate in one of our clinical studies, we explain how we may provide continued treatment for patients after their study treatment, or after the whole study, has completed
2. Where patients are suffering from a disease that is not possible to be treated with existing medicines, or patients are not able to participate in one of our clinical studies, we explain how we handle requests for compassionate or emergency use of our new medicines

### 3. POLICY

#### 3.1 Criteria

Where there is a possible need for patients to gain early access to a new medicine that is not generally available for prescription, COMPANY NAME will consider the need for early access using the following criteria:

- Where it is expected that there is a need for ongoing treatment with one of our new medicines, we recognize that this must be through normal health services once the new medicine is available for prescription. As such we will only conduct clinical studies and provide early access outside of studies, in those countries where we intend to make the medicine available through normal prescription channels after its approval.
- We recognize that we must have a minimum level of evidence that the new medicine is likely to work before considering early access and must not provide excessive risk to any patients who may receive the new medicine early.

- While addressing this patient need, we will ensure that if we agree to provide early access to our new medicines, that there is sufficient medicine available to ensure the supply will not run out for those patients receiving it early, or for those patients in our clinical studies. This is because we want to ensure that when providing early access, it does not prevent us completing clinical studies and regulatory approval that would lead to wider access through normal routes of prescription.

Any early access programs we provide will be in line with the laws and requirements of the country involved.

### *3.2 Continued treatment after clinical studies*

To be sure that our new medicines will work effectively and be safe enough to be prescribed for the diseases patients are suffering from, we study them rigorously to see whether they provide adequate relief. The results of these clinical studies allow the regulatory authorities to independently assess whether to allow the new medicines to be prescribed. As this can take several years, patients who participate in our clinical studies may have to wait for a new medicine to become available on prescription.

Where there may be a benefit for a patient to continue to be treated after the clinical study has ended, but before it is available on prescription, we will decide if we have enough information to justify early access. This will depend on the severity of the disease we are studying, how much evidence we have that the medicine works as expected and is sufficiently safe, and whether alternative effective medicines are already available on prescription. If we plan for continued treatment after a clinical study, we will include this information in our study documents so that it is clear to each patient whether this is a possibility before they start in the study.

We understand that it can be difficult to decide to participate in a clinical study, so will ensure we make this information as clear as possible. To be sure we can assess whether the new medicine continues to work effectively and is safe enough to continue to be used during early access, we will ask for the patient's consent for their physician to collect some relevant information from them on an ongoing basis while they receive the medicine.

### *3.3 Compassionate or emergency use outside of clinical studies*

The best way to see if a new medicine is sufficiently safe and effective is to run clinical studies. As such we prefer that, where possible, patients have the opportunity to participate in our clinical studies. We recognize however that not everyone has this opportunity. Where patients who cannot enroll in a clinical study are suffering from serious or immediately life threatening diseases, and there are no satisfactory alternative treatments available, we will consider early access if there is enough evidence available in their condition (as described above) and whether there is a possibility of them being able to access the medicine on prescription in the future.

In the same way as after a clinical study, we want to be sure we can assess whether the new medicine continues to work and is safe enough to continue to be used. As such we will ask for the patient's consent for their physician to collect some relevant information from them on an ongoing basis while they receive the medicine.

### *3.4 Discontinuation of early access*

When a new medicine receives approval from the regulatory authorities for the disease we are studying, and is available for prescription in the patient's country, we will phase out the early access to allow the health system to prescribe the new medicine in the normal manner.

If however the medicine is approved but not available for prescription for some reason, and there is no alternative medicine available, we will work with country authorities to discuss if we can jointly set up a patient-assistance program to allow patients who are benefitting from it, who do not have alternative treatment options, to continue to receive the new medicine. Patient safety is our primary concern, and we respect patient safety by continually evaluating all the evidence that we have available to ensure that early access use of new medicines remains effective and appropriately safe. If however the evidence demonstrates that the new medicine is not going to be as sufficiently effective or safe as we had hoped, we will discontinue all early access and work with patients and their physicians to help them safely transition patients to other treatments. When regulatory authorities do not approve the use of the new medicine for prescription, because it is considered insufficiently safe or effective, and COMPANY NAME discontinues its work to achieve regulatory approval, we will work with patients and their physicians to help them safely transition patients to other treatments.