



Roche Position¹ on Pre-Approval Access to Investigational Medicinal Products

Background

Roche discovers, develops, manufactures and markets medicines to treat people living with serious or life-threatening medical conditions. We seek to do this as safely and rapidly as possible. To achieve this, we conduct clinical trials to evaluate the safety and efficacy of Investigational Medicinal Products (hereinafter referred to as “new medicines”) in order to obtain clinical data that enable regulatory and reimbursement authorities to review and decide on whether these new medicines should be marketed and reimbursed thus allowing broad access for use under the supervision of a qualified healthcare professional or doctor. It may not be possible, however, for all patients who might receive benefit from the new medicine to enroll in the ongoing clinical trials, and nor is it possible to have clinical trials for all potential disease settings available.

During the process, new data may be generated indicating a substantial clinical benefit for some people living with serious or life-threatening medical conditions. The greater the degree of benefit for these people the greater the urgency to make the new medicine more widely available. Under specific circumstances, Roche may provide pre-approval access (PAA) to these medicines.

New medicines may be made available to patients through either an Expanded Access Program (in America) or other local PAA programs. Individual patient compassionate use might also be available in special circumstances. Further details of the criteria for these programs appear below, but in general, there are two approaches:

- An Expanded Access Program (EAP) or other local PAA programs are company run clinical programs which provide a framework within which the medicine is made available to a group of people who have the same specific disease or condition that was studied and which would fall within the marketing indication that is actively being pursued with regulatory authorities.
- Compassionate Use (CU) is a mechanism to provide a new medicine to an individual patient or set of individual patients who have a serious or life threatening disease or condition for which no satisfactory alternative therapy exists or who cannot enter a clinical trial.

This document summarizes Roche’s underlying principles regarding the ability of patients to receive new medicines before they have been approved by regulatory authorities.

¹ Pertains to SDGs 3 and 16



Stakeholders' Expectations and Concerns

For some people living with serious or life-threatening medical conditions the potential benefit of a new medicine justifies the potential risks of taking a medicine which has not yet received regulatory approval. In recent years, patient groups and individual patients have publicly asked for access to certain new medicines and have called on pharmaceutical companies to meet this need.

Roche Position

Roche understands the concerns that people living with serious or life-threatening conditions have about accessing new medicines as soon as possible. Roche is therefore committed to putting in place arrangements so that people with serious or immediately life threatening diseases or conditions, for which no satisfactory alternate treatments are available may have access to new medicines outside of on-going clinical trial programs and before such new medicines receive regulatory approval and are commercially available.

Various regulatory mechanisms exist in different countries to provide pre-approval access to new medicines and as a result, regional variations in pre-approval access will occur. Any pre-approval access to new medicines must always comply with the applicable country-specific laws and regulations, including medicine importation requirements.

In general, we believe that patients receive the best access and care when they participate in clinical studies, and this is the best way to access our new medicines. When this is not possible, and when supported by strong clinical data, we may put in place a specific program to meet these needs – an EAP (in the US) or other local PAA programs. Because of our broad clinical programs and the multiple treatments generally available to patients, we anticipate that requests for access to our new medicines through CU will be uncommon.

We also believe that the involvement of the patient's own doctor is a very important part of this process and all of our programs are conducted and supported by doctors well qualified in their respective areas. We do not charge patients for the cost of the new medicine prior to marketing approval, and we will not discontinue any patient who is participating in this program based on their ability to pay for the medicine after marketing approval.

All inquiries will be evaluated in an unbiased fashion and decisions will be based entirely on the patient's clinical condition in the context of the clinical data available. Roche clinical teams will work closely with the patient's qualified doctor and this will be the primary communication channel.

Pre-approval access may be limited in scope due to limited resources (e.g., whether there is sufficient new medicine supply available to support pre-approval access). Mechanisms to fairly allocate limited new medicine supply may be used as needed.

Additional criteria are outlined below, and all of these must be met before we will consider granting pre-approval access to a new medicine.

Roche will consider granting pre-approval access to a new medicine prior to approval only when ~~all~~ all of the following criteria are met:

- The patients for whom PAA is sought have a serious or life threatening disease or condition.
- The potential patient benefit justifies the potential risks of the new medicine use and there is a clear biological rationale to anticipate that the patient will benefit from treatment.
- The medicine requested must be part of an active and ongoing development program within the Roche group.
- The PAA must not interfere with, or in any way compromise, the conduct of any ongoing or planned clinical trials that could support health authority approval of the medicine or future commercial access to the molecule:
 - Patients for whom PAA is requested must not be eligible for ongoing clinical studies or studies that will be about to start.
 - Geographic limitations to participation in a clinical trial of the new medicine would generally not meet this criterion.
- Considerations to special safety risks must also be present:
 - Patients with exceptional safety risks that have not been sufficiently studied (e.g., patients with renal failure, heart failure or other similar co-existing conditions) should be excluded.
 - There is sufficient clinical data to identify a recommended dose that might be effective and is reasonably safe.
 - Similarly, there is sufficient clinical data to identify an appropriate dose for use in children, if the request is for a child.
- For a group of patients (EAP (US) or other local PAA programs):
 - The PAA is limited to groups which have been shown to derive benefit based on sufficient clinical data and who have the specific disease or condition that would fall within the marketing indication that is actively being pursued with regulatory authorities.
 - Whether or not other therapies to treat the disease or condition may be available, the benefit of the test medicine in the main clinical study being submitted to the regulatory authorities must be robust and persuasive.

- For an individual patient (CU):
 - The patient must have already exhausted **all** therapies typically used to treat the disease and is no longer responsive to, or able to tolerate, these treatments.
 - No other viable therapies or clinical trials are available.
 - The request for the medicine must come from the patient's qualified physician or the country's Health Authority.

For further information about how this policy is being implemented in your local area please contact the medical information team in your country or submit your question using the Roche PAA Contact Form found on [Roche's access to investigational medicines page](#). A list of all the Roche affiliates can be found on [www.roche.com/roche worldwide](http://www.roche.com/roche_worldwide).

This position paper was proposed by the Corporate Sustainability Committee and adopted by the Corporate Executive Committee on May 13, 2013 and entered into force the same day.

It was reviewed in April 2020.