EMD Serono Position Statement
On Early Access to Experimental Medicine
(also know as Compassionate Use and Named Patient Programs in various countries)

For External Use

Why it matters?

Patients facing life-threatening, chronic or seriously disabling illnesses or diseases live with the hope that tomorrow will bring a new medicine to extend and improve their lives. EMD Serono is committed to developing new medicines for patients with such illnesses and diseases. EMD Serono believes the best way to fulfill this commitment is to conduct clinical studies to assess the safety and effectiveness of experimental medicines which, if proven, will allow us to obtain drug approval from regulatory authorities and provide patients with broad access to these new medicines. EMD Serono believes that patients should be encouraged to participate in clinical studies, the goal of which is to secure approval of new, safe and effective medicines for the broadest number of patients.

EMD Serono also understands that patients with life-threatening, chronic or seriously disabling illnesses and diseases may be ineligible or unable to participate in a clinical study and may want access to the experimental medicine being tested in the clinical study. EMD Serono also understands that patients may want the medicine after a clinical study ends but before marketing authorization is granted. These different scenarios are covered by EMD Serono's **Early Access Program**.

Although these types of programs are known by many different names around the world – such as Named Patient Programs (EU), Named Patient Supply (EU), Compassionate Use (EU and various countries), Single Patient IND (US), Authorisations Temporaires d'Utilisation (France), Special Access Programs (Canada), Named Patient Access (Japan), and Special Access Scheme (Australia) – "Early Access" is the comprehensive term EMD Serono uses to encompass all of them.

EMD Serono encourages patients to consult their physicians for determining the best course of action depending on their individual needs.

EMD Serono Position Statement

EMD Serono will consider providing patients with access to an experimental medicine outside of a clinical study in accordance with applicable laws and regulations using the following principles:

1. The patient has a life-threatening, chronic or seriously disabling illness or disease. In order for early access to be appropriate, the patient should have a life-threatening, chronic or seriously disabling illness or disease, as defined by the local laws and regulations applicable to the particular patient. In addition, the patient should have exhausted all available therapeutic options typically used to treat the illness or disease, or should no longer be able to tolerate such treatments.

2. The experimental medicine should be under active clinical development. The experimental medicine should be under active clinical development for EMD Serono to support early access to it. Once clinical development of an experimental medicine has been discontinued for any reason, that medicine will not, as a general rule, be made available through EMD Serono's Early Access Program, except for patients enrolled in clinical studies with that medicine prior to the discontinuation.

3. The patient is ineligible for, or otherwise unable to participate in, clinical studies. A patient seeking early access to an experimental medicine generally should not be eligible for participation in a clinical study. Geographic limitations alone would generally not be considered a barrier to participation in clinical studies.

4. The potential benefit to the patient should outweigh potential risks. The potential benefit to the patient seeking early access should generally outweigh the combined potential risks of the experimental medicine and the outcome of the illness or disease itself. To support the use of the experimental medicine in medical treatment of the patient, there should be sufficient evidence of efficacy to expect that the individual may derive a clinically meaningful benefit. Alternatively, for molecularly targeted agents, there should be compelling biological rationale for that disease coupled with adequate human clinical data to support an assessment that the potential patient benefit justifies the potential risks of the treatment use, and those potential risks are not unreasonable in the context of the disease or condition to be treated. In addition, there must be sufficient evidence to expect that the experimental medicine will have an acceptable safety profile for the intended patient population.

5. Any early access should not jeopardize the ongoing clinical development program. The successful completion of the clinical study process is necessary to demonstrate that an experimental medicine is safe and effective for its proposed indication, which is required to obtain regulatory approval by health authorities, and to make the new medicine available to the broadest patient population appropriate. Thus, granting access to an experimental medicine through an early access program should not delay, interfere or compromise the completion of clinical studies that are intended to support approval by regulatory authorities. For example, EMD Serono will consider the availability of adequate supplies of the experimental medicine for ongoing or planned clinical studies and potential impact on the availability of approved medicines.

6. Providing patients with early access to experimental medicines requires careful consideration. To determine whether access to an experimental medicine is the best possible treatment option for an individual patient, the request for early access should generally come from the patient's qualified physician. Because experimental medicine has not yet been determined to be safe and effective, regulatory authorities and institutional review boards/ethics committees (as applicable) should generally approve early access to an experimental medicine, as required by applicable law, before EMD Serono provides the experimental medicine to a patient.

7. Enhancing Information about Early Access Programs. The development of innovative, safe and effective medicines for life-threatening, chronic or seriously disabling illnesses or diseases represents an urgent and unique challenge that requires special attention. EMD Serono is committed to continuing its work with patients, patient advocacy groups, regulatory authorities, healthcare practitioners, academia and policymakers to help ensure that there are appropriate and targeted regulatory approaches to accelerate the development and availability of innovative new medicines for patients. For additional information, click here.