EMD Serono Position Statement on Early Access to Experimental Medicine

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 **as safely and quickly as possible**.

EMD Serono believes the best way to fulfill this commitment is to conduct clinical studies to assess the safety and effectiveness of experimental (investigational) medicines which, if proven, will allow us to obtain marketing approval from the US Food and Drug Administration (FDA) and provide patients with broad access to these new medicines.

EMD Serono believes that patients who desire access to experimental medicines 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. During the clinical study process, new data may be generated indicating a substantial clinical benefit for some patients. The greater the degree of benefit for these patients, the greater the urgency to make the new medicine more widely available.

Although many patients may gain access to an experimental medicine during clinical studies, EMD Serono understands that some patients may not be eligible or able to participate in a clinical study as clinical studies may not be open for enrollment. Those patients may also want access to the experimental medicine being tested before its approval.

Early Access mechanisms offer ethical, compliant, and controlled means of pre-approval access to experimental medicine outside of clinical studies for patients with life-threatening, chronic or seriously disabling illnesses or diseases for which no satisfactory alternative therapy exists.

Although these mechanisms are known by many different names including Expanded Access, Compassionate Use, Single Patient IND Access, and Right to Try Access, "**Early Access**" is the comprehensive term EMD Serono uses to describe all of them.

Because Early Access mechanisms operate outside of a clinical study, Early Access is generally not intended for study participants who would otherwise be eligible for continued or post-study access to the experimental medicine after completing a study.

EMD Serono encourages patients to consult their physicians to determine the best course of action given their individual needs.

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as amended, Version 3.0

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 federal and state laws and regulations using the following 5 principles:

- Principle 1 - The patient has a life-threatening, chronic or seriously disabling illness or disease.For Early Access to be appropriate, the patient should have a life-threatening, chronic or seriously disabling illness or disease, as defined by federal and/or state laws and regulations. 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. EMD Serono may also require certain documentation, including, but not limited to, a patient's informed consent and certification from the patient's physician concerning the patient's eligibility for Early Access if this is required under federal or state laws and regulations.
- Principle 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 Early Access. Depending on the kind of Early Access sought and the applicable laws and regulations, additional criteria may also need to be met for the experimental medicine to be provided. For instance, for certain kinds of access in the US, the experimental medicine must have completed a Phase I clinical trial and be under investigation in a clinical trial that is intended to demonstrate the experimental medicine's effectiveness.
- Principle 3 - The patient is ineligible for, or otherwise unable to participate in, a clinical study. A patient seeking Early Access to an experimental medicine generally should not be eligible for participation in a clinical study. As explained above, EMD Serono may require a certification from the patient's physician concerning the patient's eligibility for Early Access if this is required under local laws and regulations.
- Principle 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 forthat 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 or patient population.

- Principle 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 marketing approval by the FDA, 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 marketing approval by the FDA. For example, EMD Serono will consider the availability of adequate supplies of the experimental medicine for ongoing or planned clinical studies, as well as future commercial access to the medicine once approved. Even if access is approved by EMD Serono, access may be terminated under certain circumstances, such as new study findings or experimental medicine supply limitations.

Providing patients with Early Access to experimental medicines requires careful consideration. EMD Serono is committed to evaluating all requests in a fair and equitable manner. 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 treating physician. EMD Serono may require more detailed information in order to fully evaluate a request. The requesting physician must agree to obtain appropriate FDA and IRB committee approvals and comply with regulatory obligations, including obtaining patient consent, patient monitoring and safety monitoring.

Each request will be given careful consideration by EMD Serono. There is no guarantee that Early Access to an experimental medicine will be provided. There may be circumstances that are not stated above which weigh against providing such access. Accordingly,whether EMD Serono provides Early Access to an experimental medicine will be decided on a patient-by-patient basis and its decisions are final.

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 itswork with patients, patient advocacy groups, the FDA, healthcare professionals, academia and policymakers to help ensure that there are appropriate and targeted regulatory approaches to accelerate the development and availability ofinnovative new medicines patients.

How to Request Early Access

Physicians seeking pre-approval access for patients who have exhausted all available therapeutic options typically used to treat the illness or disease, or who no longer is able to tolerate such treatments, should submit their requests to earlyaccess@emdserono.com.

We regularly monitor this mailbox and will use our best efforts to acknowledge each request within 3 business days after receipt.