Requests for Expanded Access to Investigational (Unapproved) Drugs

This Policy for Requests for Expanded Access to Investigational Drugs describes the principles and procedures that Orbus Therapeutics, Inc. ("Orbus" or "the Company") will follow when considering requests by licensed physicians for use of Orbus's investigational drugs outside of clinical trials in accordance with the requirements of the 21st Century Cures Act and other legal and regulatory obligations. Please see below for further details. "Expanded Access" is also sometimes referred to as "Compassionate Use" or "Preapproval Access". This policy may be revised by Orbus at any time.

Orbus Policy for Evaluating Expanded Access to Investigational Drugs not Provided Through Orbus Clinical Trials

Orbus currently is testing in clinical trials investigational drugs that have not yet been approved by the US Food and Drug Administration (FDA) for commercial sale. We have conducted and are conducting research on our investigational drugs so that we can better understand how these investigational drugs work, obtain proof that they are safe and effective, and gain approval from FDA and other international regulatory authorities to make these drugs available commercially.

On rare occasions, physicians may identify patients with serious diseases or conditions who cannot participate in our clinical trials but who may benefit from one of our investigational drugs despite its lack of demonstrated safety and effectiveness. In such situations, Orbus will on a case by case basis consider requests from physicians for a supply of investigational drug to use with a specifically identified patient.

Orbus will evaluate such requests in a scientifically and ethically responsible way according to the principles and procedures set forth below and applicable government regulations.

1. The investigational drug must be in active clinical development. Orbus must be actively studying the investigational therapy in Phase 2 or Phase 3 clinical trials in the United States conducted under an investigational new drug (IND) application filed with the FDA or in Phase 2 or Phase 3 clinical trials in the European Union under an equivalent application filed with the European Medicines Agency. In considering applications for and providing expanded access therapy to patients outside the United States, Orbus is required to abide by local government laws and health authority regulatory guidelines.
2. The patient must have a serious or life-threatening disease or condition.
3. The request for use of the investigational drug must be within the requirements set forth in FDA regulations and must be within the scope of Orbus's current research interests as determined by Orbus in its sole discretion, including but not limited to, uses being studied in Orbus's clinical trials or prior Phase II or III trials conducted by Orbus or the US National Cancer Institutes.
4. Patients must be ineligible for participation in ongoing clinical trials of the investigational drug. If, however, it is clear that a patient does not meet the criteria for participation in the clinical trial or is unable to participate in the trial for geographic reasons, this requirement may be waived.
5. After meeting the needs of clinical trials and other patients, Orbus must have a sufficient supply of the investigational drug to reasonably accommodate the likely duration of treatment.
6. Providing expanded access to the investigational new drug must not interfere with ongoing clinical trials or regulatory submissions.
7. There must be a positive benefit-risk ratio for the patient based on the treating physician's medical judgment. The potential benefits to the patient seeking access to the investigational drug must always outweigh the collective potential risks to the patient.

Procedure for Requesting Expanded Access and Response Times

1. Physicians interested in treating a patient with an Orbus investigational new drug that is in active clinical development must fill out the FDA Individual Expanded Access Application for FDA3926 and acknowledgement: Information on obtaining form FDA3926 can be found here. This form must be submitted to Orbus for review using the instructions below. The physician must be properly licensed where the patient will be treated.
2. When the form has been satisfactorily completed and submitted to Orbus, the Company aims to acknowledge receipt via email within 72 hours of submission of the request.
3. Submitting a request does not guarantee that expanded access to an investigational drug will be granted, even if all of the above criteria are met. Orbus will review each request on a case-by-case basis and will usually make a decision to grant or deny the request, or ask for more information, within 10 business days. The decision to grant expanded access is solely Orbus's decision.

Contact for Further Information

Persons with questions about Orbus's policy and procedures for expanded access or about expanded access to Orbus investigational drugs may send an email to the following address: info@orbustherapetuetics.com.

Further information about Orbus's clinical trials is available on Orbus's website and on the NIH's ClinicalTrials.gov website.