Innate Immunotherapeutics

Innate Immunotherapeutics Limited (Company)

Compassionate Use Policy

Innate Immunotherapeutics (IIL) is focused on developing a treatment for patients with the chronic disabling progressive form of multiple sclerosis. To do this, we conduct clinical trials to assess the safety and efficacy of our drug candidate(s), which if proven, will allow us to obtain the necessary approvals from regulatory authorities to provide patients with broad access to these medicines.

In general, IIL believes that participating in clinical trials is the best way for patients to access medicines prior to approval. In some extreme circumstances, when this is not possible, patients with significant debilitating diseases or conditions may seek special access to investigational unapproved medicines outside of a clinical trial setting. These situations are typically referred to as compassionate use, but can also be known as expanded access, early access, pre-approval access and emergency use.

The clinical development process (which is the process by which a drug is developed and tested for safety and efficacy, and if proven, submitted to regulatory authorities for approval for use) involves controlled testing in humans to ensure both safety and efficacy. Because it is not known in clinical development whether an investigational medicine is safe or effective, compassionate use may present numerous risks for the patient and for the clinical development program. For patients, compassionate use may bring potential safety risks or a false sense that the medicine will provide benefit; for the clinical development program, it can delay or jeopardize the approval of a new medicine sought by many.

Conducting clinical trials is extremely complex and challenging. The ultimate goal is the rigorous testing of the clinical product with the aim of securing regulatory approval and enabling the medicine to be available to as many patients as possible as quickly as possible. IIL has ethical responsibilities to ensure the quality and integrity of clinical trials and to minimize risks to current research participants and future patients. These ethical responsibilities require that appropriate criteria are applied for compassionate use of our investigational medicines.

IIL considers many factors when considering a request for compassionate use of an investigational medicine, such as the strength of the clinical data, the benefit-risk profile, the impact on the clinical development program, the phase of development, and probability and timing of regulatory approval.

At IIL, a compassionate use program, or a single request for compassionate use of an investigational medicine, can only be considered if all of the following conditions are met:

1. The disease or condition being studied is seriously debilitating and/or life threatening.

2. There are no adequate alternative therapies or clinical trials available.

3. Sufficient preliminary efficacy and safety data exist for the drug in order for IIL to make a benefit-risk analysis consistent with the establishment of a compassionate use program.

4. Sufficient clinical data is available to identify an appropriate dose.

5. A patient's treating physician and IIL's Medical Consultant both believe there is the potential for the specific patient under consideration to reasonably expect benefit from the treatment, and there is robust evidence to support the possibility that the patient will benefit.

6. Adequate supply exists to support both the ongoing clinical trials and approved compassionate use, until and if product becomes commercially available.

7. The patient is not eligible or a candidate for one of the IIL-sponsored studies on the therapy.

8. Compassionate access will not adversely impact the clinical development program, in particular, the conduct of a pivotal clinical trial that is required for regulatory approval.

9. The request must be made by the patient's treating physician in compliance with local law and such law must allow for the use of an experimental unapproved medicine at the discretion of the physician.

The above criteria are those that IIL will consider in determining whether to offer compassionate use; however, IIL cannot make a guarantee that a compassionate use program will be available, and, even if a compassionate use program is offered, IIL cannot make a guarantee that the investigational medicine will be available to a particular patient.

If all these conditions are met, IIL will consider compassionate use requests from treating physicians subject to local/national laws and regulations. All requests will be evaluated in a fair, unbiased manner. Patients with exceptional safety risks that have not been sufficiently studied would be excluded. Any pre-approval access to investigational product must always comply with the applicable country-specific laws and regulations, including medicine importation requirements. If approved, the patient must provide written informed consent to the treating physician and also consent to comply with the safety and monitoring requirements defined by IIL. The treating physician must also agree to comply with the safety and monitoring requirements. Compassionate use will cease being made available if, as a result of clinical trials, the product does not demonstrate a positive risk benefit to patients.

This Policy was adopted by Innate Immunotherapeutics Limited Board on 17 July 2014.