# Compassionate Use & Pre-Approval Access

The well-being of patients is the reason we come to work each day. Our goal is to help them by developing effective and safe medicines.

We are often asked how patients with serious diseases can get medicines not yet approved by government health authorities. These medicines are still being studied and are also called investigational medicines. There are a plethora of terms used; however pre-approval access (PAA) is the overarching term for any access prior to approval, including single patient requests (compassionate use) and planned early access programs. In the United States, the government health authority is the U.S. Food and Drug Administration (FDA).

While government health authorities have made great advances in shortening the time it takes to make new investigational medicines available, some seriously ill patients are still in urgent need of new treatment options. We understand that urgency, especially for patients with medical needs that are not met by treatments available now.

We follow five important principles when providing pre-approval access to investigational medicines:

1. All requests for pre-approval access are considered in a fair and just manner;

2. Sufficient understanding of the potential benefits and risks of the investigational medicine has been established through the conduct of a rigorously designed, scientifically and medically sound, development program;

3. Patients are not put at risk of unnecessary harm;

4. Fulfillment of pre-approval access will not jeopardize the development program that may lead to broader public access through marketing authorization; and

5. Fulfillment of pre-approval access fully complies with applicable laws and regulations.

Circumstances in which Pre-approval Access Programs and Single Patient Requests may be considered:

1. The patient must have a serious or life-threatening disease or condition.

2. There must be an unmet medical need, or alternative therapies are not available, or the patient must have exhausted all such alternative therapies.

3. The patient is not eligible or cannot participate in a clinical trial. In assessing the eligibility of a patient for potential pre-approval access, preference will be given to clinical trials, then pre-approval access programs, and then single patient access

4. Sufficient scientific evidence to demonstrate that the benefits of the investigational medicine outweigh the risks.

5. Providing pre-approval access will not jeopardize the initiation, conduct, or completion of clinical investigations and the overall development program to support registration of the product.

6. Pre-approval access must be permitted by, and run in accordance with, applicable laws.

7. The treating physician making the request is licensed and qualified to administer the investigational medicine, and agrees to comply with Janssen requirements and local regulations governing pre-approval access, and adhere to applicable laws and regulations.

For additional information on Compassionate Use & Pre-Approval Access, click here.

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