

# Position on Pre-Approval Access

## Background

Pre-approval access (PAA) is the overarching term for any access prior to approval, including single patient requests, or cohort programs. Expanded Access (EA) refers to treatment options that allow the use of a medicine that has not been authorized by the relevant national health authority outside of a clinical trial setting. Such use of medicines in development (investigational medicines) may be made available to seriously ill patients in cases where there are no satisfactory authorized therapies or clinical trials for which they are eligible. In order to provide relief to patients with serious or terminal diseases, or those for whom extreme suffering could be alleviated, PAA is an important treatment pathway, provided it is used ethically and responsibly.

## Relevance

As the largest, most diversified healthcare products company, Johnson & Johnson is a leader in healthcare research and development (R&D). While we recognize the need for strict controls regarding approval of medicines before making them available, we also believe that the benefits we can bring to certain patients through investigational medicines often outweigh the risk to them. Within clear rules and guidelines, we support helping patients through PAA and EA.

## Guiding Principles

As stated in [Our Credo](#): “We believe our first responsibility is to the patients, doctors and nurses, to mothers and fathers and all others who use our products and services. In meeting their needs everything we do must be of high quality.” This acts as a constant guide to our decision-making and reinforces that improving healthcare for the people we serve—patients and customers—must come first as we seek to break new ground with advances in science and technology.

Our [Ethical Code for the Conduct of Research and Development](#) provides more specific standards of conduct and behavior for physicians, clinical research scientists and others who are responsible for medical aspects of R&D.

## Our Position

We believe that it is right to offer solutions to assist patients in urgent need of new treatment options where no applicable feasible treatments currently exist. While we support the intentions of government health authorities to reduce the time required to make new investigational medicines available, in certain cases, we understand that investigational medicines may be the only available recourse. We therefore support PAA and

EA, in line with ethical and regulatory guidelines. We follow five important principles when providing PAA access to investigational medicines.

### **Principles for PAA at Johnson & Johnson**

1. All requests for PAA 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 PAA will not jeopardize the development program that may lead to broader public access through marketing authorization.
5. Fulfillment of PAA fully complies with applicable laws and regulations.

### **Conditions for consideration of PAA and Single Patient Requests**

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 PAA, preference will be given to clinical trials, then PAA programs, and then single patient access.
4. Sufficient scientific evidence to demonstrate that the benefits of the investigational medicine outweigh the risks.
5. Providing PAA will not jeopardize the initiation, conduct, or completion of clinical investigations and the overall development program to support registration of the product.
6. PAA 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 PAA and to adhere to applicable laws and regulations.

For additional information on PAA, visit the [website](#) of Janssen Pharmaceutical Companies of Johnson & Johnson.

## **Application**

This Position is relevant for all pharmaceutical and medical R&D activities of the Johnson & Johnson Family of Companies, as detailed in our [governance materials](#).

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