Janssen Policy
Evaluating and Responding to Pre-approval Access Requests for Investigational Medicines

PURPOSE
This policy establishes the principles by which the Janssen Pharmaceutical Companies of Johnson & Johnson evaluate and respond to requests for pre-approval access to investigational medicines outside of a clinical trial.

PRINCIPLES FOR PRE-APPROVAL ACCESS REQUESTS
At the Janssen Pharmaceutical Companies of Johnson & Johnson (Janssen), our Credo values guide our decision-making. We adhere to these values and we strive to maintain the highest ethical standards in our scientific research methods and programs. We also comply with regulatory and industry guidelines as we seek to make advances in science and technology.

We are often asked how patients with serious diseases may obtain medicines not yet approved by government health authorities (investigational medicines). Pre-approval access (PAA) is the overarching term for any access to an investigational medicine outside of a clinical trial, and before it is approved by government health authorities.

The main pathway for gaining access to Janssen's investigational medicines is for a patient to enroll in a clinical trial. For patients who cannot enroll in clinical trials, pre-approval access may be considered.

Our policy for considering pre-approval access to investigational medicines is grounded in key ethical principles, including that:
1) All requests for pre-approval access are considered in a fair and just manner;
2) There is sufficient understanding of the potential benefits and risks of the investigational medicine;
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

SCOPE
This policy applies to investigational medicines that are being developed under Janssen's sole control, or in partnership with another company. In cases where an investigational medicine is developed in partnership with another company, Janssen may not control decisions regarding pre-approval access.

This policy describes the pathways for pre-approval access to investigational medicines and the processes for requesting, and:
- contact information for Janssen;
- the procedure to make pre-approval access requests;
- the criteria Janssen uses to evaluate pre-approval access requests;
- the time it will take to acknowledge the receipt of such a request; and
- references to Janssen clinical trial and pre-approval access information & global pricing policy for requests from self-pay markets outside of the United States

This policy does not apply to requests related to affordability or failure to be reimbursed (e.g., private insurer).

Decisions on pre-approval access are some of the most difficult that any company can face. Janssen will consider each request carefully but cannot guarantee that pre-approval access will be granted in any particular case.

POLICY STATEMENTS
Pathways for Access to Investigational Medicines
When evaluating a request for pre-approval access, the patient's eligibility for clinical trials must first be considered, then the patient's ability to participate in **any** available pre-approval access *programs*, and finally through pre-approval *single patient access*.

Clinical Trials
The main pathway for gaining potential access to Janssen's investigational medicines is to enroll in a clinical trial. Clinical trials are scientific studies in which investigational medicines are tested to assess whether they are safe and effective.

Sometimes patients are not eligible to enter a clinical trial. Reasons for this can include if the patient has other health conditions in addition to the type of disease being studied, or if the patient is taking certain other medicines that might interfere with the trial results or put the patient at unreasonable risk.

Pharmaceutical companies, like Janssen, conduct clinical trials to establish the scientific proof needed to ask a government health authority to approve our medicines. Obtaining that approval is critical because it means that an independent health authority has reviewed all of the efficacy and safety information on the medicine and believes that it should be made available for physicians for prescribing to patients.

Pre-Approval Access Programs
Patients may sometimes obtain access to an investigational medicine through a single patient request or a pre-approval access program. These programs are designed for a group of patients by which pharmaceutical companies may provide certain investigational medicines outside of a clinical trial before those medicines have been approved by the government health authority in the country in which the patients live. Similar to clinical trials, pre-approval access programs may have specific criteria that patients must meet to be included. Such requests also need to be approved by government health authorities.

Pre-approval Single Patient Access
Single Patient Access (SPA) is a pre-approval access pathway in which access to an investigational medicine may be considered for an individual patient in situations where (i) the patient does not meet the eligibility criteria for clinical trials and (ii) is not able to participate in any type of pre-approval access program. Single patient access is also known as “compassionate use” and is also regulated by local health authorities.

Right to Try (RTT)
Right-to-try laws are U.S. state laws that were enacted to permit terminally ill patients to try experimental therapies (drugs, biologics, devices) that have completed Phase 1 testing but have not been approved by the FDA. In this setting, the company from whom the drug is being requested must approve each request. Federal legislation is pending that, if enacted, would provide for a similar mechanism.

Janssen fully supports efforts to improve the safe and timely access to investigational medicines for patients whose chronic or terminal illnesses are not responding to currently available treatments. We are guided by a priority to ensure the safety of all patients and believe every member of the public, including patients seeking access to investigational medicines, deserves the reassurance of knowing that safeguards are in place to protect public health and ensure the safety of all medicines.

Our own innovation in this area, developed in collaboration with the NYU Division of Medical Ethics has been the Compassionate Use Advisory Committee. We believe that this is a fair, ethical and streamlined process for patients seeking access to our investigational medications, while still offering a thorough consideration of safety.

We are committed to helping patients with serious illnesses and their families request access to our investigational medicines. We support these requests through our established review and evaluation processes, which includes independent review by the FDA to assure full consideration of available safety data of which the FDA may be uniquely aware. In addition to internal review, these requests may also be reviewed by the Compassionate Use Advisory Committee (CompAC) to support our commitment to fair and equitable evaluation.

Circumstances in which Pre-approval Access Programs and Single Patient Requests May be Considered
For both Pre-approval Access Programs and Pre-approval Access Single Patient Access, Janssen considers pre-approval access to an investigational medicine, when all the following circumstances can be confirmed:
• The patient must have a serious or life-threatening disease or condition.

• There must be an unmet medical need, or alternative therapies are not available or the patient must have exhausted all such alternative therapies.
• 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.
• There is sufficient scientific evidence to demonstrate that the benefits of the investigational medicine outweigh the risks.
• 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.
• Pre-approval access must be permitted by, and run in accordance with, applicable laws.
• 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.

Procedure to Submit a Request and obtain an Acknowledgement of Receipt
1. How do I submit a Pre-approval Access request to Janssen?
Requests must be submitted by the treating physician caring for a patient.
Physicians must submit a PAA request by registering for access to the Janssen Global Tracking System https://www.janssenmanagedaccess.com
Janssen will not accept requests directly from patients.

2. Does Janssen accept Right to Try requests?
We are committed to helping patients with serious illnesses and their families request access to our investigational medicines. We support these requests through our established review and evaluation processes, which includes independent review by the FDA to assure full consideration of available safety data of which the FDA may be uniquely aware. In addition to internal review, these requests may also be reviewed by the Compassionate Use Advisory Committee (CompAC) to support our commitment to fair and equitable evaluation.

Physicians in the United States wishing to submit a Pre-approval Access request should call 1-800-JANSSEN or email: janssenmedinfo@its.jnj.com. Physicians outside of the U.S. should contact the Janssen office in their country. Janssen does not accept requests directly from patients or family members.

For additional guidance and assistance with submitting requests to the FDA as well as information about IRB submissions, please visit:
https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm

http://navigator.reaganudall.org/

3. When will an acknowledgement of receipt be received?
For physicians submitting a formal Pre-approval Access request to Janssen's Managed Access Portal, an acknowledgement of receipt will immediately be issued upon submission completion.

4. What happens if further medical information is needed from the requesting physician?
For physicians submitting a Pre-approval Access request to Janssen, it is possible that they will be asked to provide further medical information. Responding quickly to any further information requests will enable Janssen's timely evaluation of requests.

5. When will a decision about a Pre-approval Access request from Janssen be available?
We commit to working to provide a decision as quickly as possible, ideally within 5 to 10 business days, **once all required medical information is provided**. This timeline may be impacted by factors such as requests for further medical information, clinical trial processes, national and local requirements, and government health authority feedback.

How Pre-Approval Access Requests are Evaluated
Janssen's clinical personnel will conduct a review of all requests for pre-approval access. If, in Janssen's medical assessment, a patient has exhausted all available treatment options and is not eligible for clinical trials, pre-approval access may be considered. Janssen may utilize the Compassionate Use Advisory Committee (CompAC) to support its decision-making process. (See information below).

Charging for IMP
Once a major market approval occurs in the world (e.g., European Medicines Agency) patients in self-pay markets may be charged for IMP. In such cases, charging will comply with local law and regulations AND the Janssen Global Pricing policies. Terms of charging must be made available to the requesting physician for communication to patient prior to any provision of investigational medicine.

The Compassionate Use Advisory Committee (CompAC)
To enhance our long-standing commitment to ethical and patient-centered decision-making, Janssen established a partnership between Janssen and the Division of Medical Ethics at the New York University (NYU) School of Medicine to form the Compassionate Use Advisory Committee (CompAC) to assist in evaluating single patient access requests. This partnership seeks to further ensure that patient's requests for investigational medicines are evaluated in the most thoughtful, ethical and fair manner. The CompAC comprises an external committee of bioethical experts, physicians and patient representatives.

Janssen may utilize CompAC to support decision-making for single patient access requests. Requests are forwarded to CompAC following the initial Janssen review and triage. CompAC evaluates these requests and provides a recommendation to Janssen. Janssen's physicians make a final decision on patient access, taking the CompAC recommendation into account.

Company Contact Information and Additional Janssen PAA Information

To obtain more information about Janssen's investigational medicines, physicians should contact Janssen Medical Information at 1-800-JANSSEN or email janssenmedinfo@its.jnj.com. Physicians outside of the U.S. should contact the Janssen office in their respective country. Country information may be found here: www.janssen.com/contact-us. Janssen will not accept requests directly from patients.

For a list of PAA programs at Janssen:
https://www.janssen.com/compassionate-use-pre-approval-access/investigational-medicines-for-compassionate-use-requests

Reference to Clinical Trial and Pre-approval Access Information
Patients should speak with their physician about their eligibility to participate in a clinical trial or other pre-approval access options. A list of Janssen clinical trials and information about U.S. pre-approval access by product can be found at www.clinicaltrials.gov.

DEFINITIONS
ClinicalTrials.gov: ClinicalTrials.gov is an online database operated by the U.S. National Institutes of Health (NIH) of publicly and privately supported clinical trials.

Compassionate Use Advisory Committee (CompAC): An external, expert advisory committee comprised of bioethical experts, physicians and patient representatives convened by NYU to evaluate and make recommendations independently from Janssen regarding overall pre-approval access (PAA) strategy as well as single patient requests for access to investigational medicinal products[1].
[1] Janssen Research and Development, LLC, has an agreement with New York University's Langone Medical Center (NYU). Under the agreement, NYU forms and administers the CompAC.

Pre-Approval Access (PAA): Provision of an investigational medicinal product outside of a clinical trial prior to its marketing authorization to treat patients with serious/life-threatening diseases or conditions, where there exists no alternative treatments or where alternative treatments have been exhausted according to local laws and regulations**.** In general, patients would first be considered for clinical trials (the primary access pathway), then pre-approval access programs including expanded access programs (EAPs,) and only then single patient requests (SPR)**.**

Pre-approval Access Program (Program): Pre-approval access provided to multiple patients with a similar disease or condition where patients meet Janssen's pre-established medical and scientific entry criteria. The patients receive treatment pursuant to Janssen's pre-defined treatment guideline or protocol.

Right to Try (RTT): Right-to-try laws are U.S. state laws that were created to permit terminally ill

patients to try experimental therapies (drugs, biologics, devices) that have completed Phase 1 testing but have not been approved by the FDA. Legislation is under review with the House of Representatives which could make RTT Federal law.

Single Patient Access (SPA): Pre-approval access provided to an individual patient in situations where the patient does not meet the pre-established medical and scientific entry criteria for clinical trials or is unable to participate in a clinical trial or any type of Pre-Approval Access Program. Pre-approval access for individual patients may be initiated after Janssen reviews and approves an unsolicited request for pre-approval access from a treating physician and after all other criteria and considerations are met.

Single Patient Request (SPR): Single Patient Request (SPR) is a pre-approval access pathway in which access to an investigational medicinal product may be considered for an individual patient in situations where the patient (i) does not meet the eligibility criteria for clinical trials, and (ii) is not able to participate in any type of pre-approval access program. Single patient access is also known as “compassionate use” or “expanded access” and is also regulated by local health authorities.