1. Purpose

To establish and describe the Office of Sponsor and Regulatory Oversight's (OSRO) Manufacturer Expanded Access Policy when the Center for Cancer Research (CCR) is the manufacturer.

2. Scope

2.1. OSRO in the Center for Cancer Research, National Cancer Institute (NCI) shall establish and control the policy.

2.2. The policy applies only to those products for which CCR is the manufacturer and has a regulatory obligation to publish a Manufacturer Expanded Access Policy.

2.3. Limitations

2.3.1. This policy does NOT apply to CCR investigator-initiated single patient trials.

2.3.2. Nothing in this policy will supersede NCI, National Institutes of Health (NIH) or Health and Human Services (HHS) requirements.

3. Responsibilities

3.1. CCR Management is committed to providing resources to meet the requirements for implementing a Manufacturer Expanded Access Policy within OSRO and supporting its continual improvement.

3.2. OSRO personnel are responsible for understanding the Manufacturer Expanded Access Policy.

3.3. The OSRO Director is responsible for establishing and maintaining the Manufacturer Expanded Access Policy.

3.4. OSRO Sponsor and Regulatory Oversight Support (SROS) Contractor staff assisting OSRO Functional Groups are responsible for understanding the Manufacturer Expanded Access Policy.

3.5. The OSRO Director with the CCR Clinical Director are responsible for assessing Manufacturer Expanded Access Requests.

4. References

4.1. FDA Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics, May 2014

4.2. 21 CFR Part 312.310 Investigational New Drug Application – Individual patients, including for emergency use

4.3. 21 CFR Part 312.315 Investigational New Drug Application – Intermediate-size patient populations

4.4. 21 CFR Part 312.320 Investigational New Drug Application – Treatment IND or treatment protocol

4.5. Food Drug and Cosmetic Act, Section 506A (also referred to as 21 United States Code 356a) – Manufacturing changes and amended by FDASIA Section 902 Breakthrough Therapies

4.6. 42 CFR Part 11.28 Clinical Trials Registration and Results Information Submission – What constitutes clinical trial registration information?

4.7. 45 CFR Part 46 Protection of Human Subjects

5. Definitions

Refer to the OSRO Lexicon.

6. Policy

6.1. Cell Therapy Products Manufactured by CCR

6.1.1. Because of the unique nature of cell therapy products, the manufacturing complexities, and the intensive patient management, CCR will not provide cell therapy products through an Expanded Access program.

6.1.2. Patient access to cell therapy products will only be through participation in an ongoing clinical trial conducted by CCR investigators.

6.1.3. To be eligible to enroll, participants will need to meet all Inclusion and Exclusion criteria for the trial.

6.2. Non-Cell Therapy Products Manufactured by CCR

6.2.1. Participant enrollment in an ongoing clinical trial involving the use of non-cell therapy products is the preferred route for patient access.

6.2.2. Under rare circumstances, CCR will provide product under an Expanded Access Program, if all the following criteria are met:

6.2.2.1. Patients must:

6.2.2.1.1. Suffer from a serious or immediately life-threatening disease or condition.

6.2.2.1.2. Have undergone appropriate standard treatments without success and no comparable or satisfactory alternative treatment is available or exists to treat the disease or condition.

6.2.2.1.3. Be ineligible for participation in any ongoing clinical study of the investigational product, which includes lack of access due to geographic limitations.

6.2.2.1.4. Have a disease for which there is sufficient evidence of a potential benefit from the use of the investigational product and the benefit outweighs the known or anticipated risks.

6.2.2.1.5. There is adequate information to support appropriate dosing for a special population for which the patient is a part of.

6.2.2.2. The patient's treating physician in the community must attest in writing that:

6.2.2.2.1. The physician is licensed and eligible to practice in the territory in which the product will be administered.

6.2.2.2.2. The physician will be the regulatory sponsor for the Expanded Access single patient protocol.

6.2.2.2.3. All regulatory requirements will be met prior to the initiation of the planned administration of the product under the Expanded Access program and per the single patient protocol procedures.

6.2.2.2.4. All requirements of 45 CFR 46 will be met prior to the initiation of the protocol.

6.2.2.3. The OSRO Director and the CCR Clinical Director have determined that:

6.2.2.3.1. The patient meets the eligibility criteria listed in Step 6.2.2.1.

6.2.2.3.2. The risk-benefit ratio for the specific patient is favorable.

6.2.2.3.3. There is an adequate supply of the non-cell therapy product available.

6.2.2.3.4. Allocation of the non-cell therapy product for the Expanded Access single patient protocol will not adversely affect ongoing research in the CCR, or any future regulatory submission for that product.

6.3. Administrative

6.3.1. CCR will post the Manufacturer Expanded Access Policy for applicable products at the Reagan-Udall Foundation web page (http://navigator.reaganudall.org/). The information on the web page will be updated regularly.

6.3.2. Information about Expanded Access will be updated in ClinicalTrials.gov if required by 42 CFR 11.

6.3.3. Contact information will be for a member of the appropriate study team.

6.3.4. Only requests from treating physicians will be considered.

6.3.5. Study teams will notify the OSRO Director within 1 business day of receipt of each request.

6.3.6. The OSRO Director and the CCR Clinical Director will determine whether a request will be granted within five (5) business days of receipt of each request and will promptly communicate the decision to the requester.

7. Change Summary

Revision Number	Effective Date	Description of Change
1	27AUG2019	New Document
2	20Jan2022	Biennial Review Step 3.4 – added Section 4 – added hyperlinks to references Updated document language as required
3	30JAN2024	Biennial review