Treatment Use of Investigational Drugs

I. Purpose

This Procedure outlines requirements for treatment use of investigational drugs at sites under the auspices of the Einstein Institutional Review Board ("IRB").

II. Scope

The following Procedure covers all treatment use of investigational drugs at sites under the auspices of the Einstein IRB.

III. Definitions

Treatment Use: The use of an investigational (not FDA-approved) drug or device for clinical care outside of a clinical trial.

IV. Procedure

Treatment use of investigational drugs requires prior IRB review (with the exception of Emergency Use).

The FDA's Expanded Access Program ("EAP") allows for treatment use of investigational drugs in certain conditions.

The FDA permits an investigational drug to be used for treatment when:

- The drug is intended to treat a serious or life-threatening disease.
- There is no comparable or satisfactory alternative drug or other therapy available to treat that stage of the disease in the intended patient population.

The FDA permits investigational drug's use for treatment purposes after sufficient data collection shows that the drug "may be effective" or does not have unreasonable risks relative to the risk of the condition of treatment. FDA describes three distinct categories of EAP based on the number of people who need access and the level of risk. Each type of expanded access requires an expanded access IND submission.

1. **Individual patient IND**, including emergency use IND (21 CFR 312.310) is commonly held by a treating physician or an investigator for treatment of an individual patient.

2. **Intermediate population treatment IND** (21 CFR 312.315) is commonly held by the sponsor (manufacturer) for use in a population smaller than a typical treatment IND or treatment protocol. The investigational drug for intermediate population treatment INDs may be in active development or may be an FDA approved drug that is unavailable or in limited supply.

3. **Large population treatment IND or treatment protocol** (21 CFR 312.320) is commonly held by the sponsor for widespread treatment use. For a large population treatment INDs, the sponsor must pursue marketing approval.

Use of the investigational drug for an individual patient IND, Intermediate size patient IND, or Treatment IND must meet all applicable FDA requirements and must be submitted to the Einstein IRB for review and approval. All applicable regulations must be met including those at 21 CFR Parts 50 and 56. The PI should submit a copy of the letter from the FDA granting the IND; Form 3926, if applicable; and all documentation required for an initial submission for IRB review.

For single-patient IND protocols that request a waiver of full-board review, a Chair, or an experienced IRB member designated by a Chair, can review and provide concurrence prior to the treatment begins in lieu of obtaining IRB review and approval at a convened IRB meeting.

V. Effective Date

Effective as of: July 1, 2020

VI. Procedure Management and Responsibilities

Einstein's Office of Human Research Affairs is the Responsible Office under this Procedure. The Executive Dean is the Responsible Executive for this Procedure. The OHRA Director is the Responsible Officer for the management of this Procedure.