

Title: <b>Expanded Access and Use of a Test Article for Compassionate Purposes</b>	*Applicable to: <b>Beaumont Health</b>	Effective Date: <b>04/03/2018</b>
Policy Owner: <b>Administrative Director</b>	Document Type: <b>Policy</b>	Last Periodic Review Date: <b>04/03/2018</b>  Functional Area: <b>IRB and Clinical Research, Research Institute</b>

**\*For This Document, Beaumont Health Includes:**

- Beaumont Corporate Shared Services
- Beaumont Hospital, Dearborn
- Beaumont Hospital, Farmington Hills
- Beaumont Hospital, Grosse Pointe
- Beaumont Hospital, Royal Oak
- Beaumont Hospital, Taylor
- Beaumont Hospital, Trenton
- Beaumont Hospital, Troy
- Beaumont Hospital, Wayne
- Beaumont Medical Group
- Beaumont Pharmacy Solutions
- Post Acute Care

**I. PURPOSE:**

The purpose of this policy is to define “Compassionate Use” and Expanded Access Protocols in research. All Expanded Access protocols require review and approval by the Beaumont Institutional Review Board (IRB) prior to human participant use at Beaumont Health.

**II. SCOPE:**

This policy applies to investigators, research key personnel, IRB members and IRB staff.

**III. GENERAL:**

The terms **emergency use** and **compassionate use** are not synonymous, according to federal regulations. Investigators must be aware of the specific standards for emergency use and compassionate use in order to avoid violating federal regulations. See IRB Policy Emergency Use of a Test Article The Food and Drug Administration (FDA) strictly regulates all use of drugs, biologics, and devices which have not been approved by the FDA for marketing. Different regulations apply to the different types of test article use, and the terminology may be confusing. Expanded access, sometimes called "compassionate use," is the use of an investigational drug, device or biologic outside of a clinical trial to treat a patient with a serious or immediately life-threatening disease or condition who has no comparable or satisfactory alternative treatment options.

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- A. **Use of Investigational Drugs** - FDA regulations allow access to investigational drugs for treatment purposes on a case-by-case basis for an individual patient, or for intermediate-size groups of patients with similar treatment needs who otherwise do not qualify to participate in a clinical trial. The regulations also permit expanded access for large groups of patients who do not have other treatment options available, once more is known about the safety and potential effectiveness of a drug from ongoing or completed clinical trials. See the link for additional information. FDA: [Development & Approval Process \(Drugs\)](#)
- B. **Use of Investigational Devices** - An unapproved medical device is typically only used in an IRB approved clinical study in accordance with the approved protocol. There may be circumstances however, under which a health care provider may wish to use an unapproved device to save the life of a patient or to help a patient suffering from a serious disease or condition for which no other alternative therapy exists. In these circumstances, patients and their physician may have access to investigational devices under one of following FDA mechanisms by which the FDA makes an unapproved device available:
1. [Emergency Use](#)
  2. [Emergency Research](#)
  3. [Compassionate Use](#) (or Single Patient/Small Group Access)
  4. [Treatment Use](#)
  5. [Continued Access](#)
- These mechanisms can be utilized during a certain time-frame in the IDE process if the criteria are met. FDA approval is required except in the case of Emergency Use. See the link for additional information: [Expanded Access for Medical Devices](#)

#### IV. **POLICY:**

The Beaumont IRB is charged with protecting the rights of all individuals participating in research and ensuring they are provided the information necessary to make an informed and voluntary decision about participation. All human participant research must be reviewed and approved by the IRB regardless of the number of participants expected to enroll in a given study. See IRB Policy [IRB Initial Review of Research Protocols](#) for specific procedures.

#### V. **CATEGORIES OF USE:**

- A. **Research Use** - The use of unapproved devices, drugs, or biologics is typically part of a clinical research trial designed to test the safety and/or efficacy of a test article. These clinical trials require prior IRB review and approval. The majority of clinical trials are conducted under an Investigational Device Exemption (IDE) or an Investigational New Drug (IND)

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exemption obtained from the FDA, which require the research protocols to be filed with the FDA prior to their start.

- B. **Emergency Use - Emergency Use** of an unapproved device, drug or biologic is intended to benefit a **single** patient involved in a life-threatening or severely debilitating situation. A progress report should be submitted to the IRB and the FDA at three months' post treatment with the IND for a single patient.
  
- C. **Compassionate Use** - The term **Compassionate Use** has often been used to refer to the provision of investigational drugs outside of an ongoing clinical trial to a limited number of patients who are desperately ill and for whom no standard alternative therapies are available. However, the term "compassionate use" does not appear in FDA or Department of Health and Human Services (HHS) regulations. It is preferable to use the names of the specific access programs when discussing the use of investigational articles outside of formal clinical trials.
  
- D. **Expanded Access - Expanded Access** typically involves obtaining a research IND or IDE for individual patient access to investigational drugs/devices for serious diseases. These mechanisms are primarily intended to give patients access to experimental drugs, biologics or devices when no comparable or satisfactory alternative treatment is available. Although the test article sponsor is expected to continue conventional clinical trials and pursue marketing approvals with due diligence, expanded access studies involve systematic use of experimental treatments. Therefore, they require the same review and approval as research, including IRB approval and FDA approval in the form of an IDE (medical device) or an IND (drug or biologic). There are varying sizes of Expanded Access IND, Single Patient, Intermediate-Size and Widespread Use. Please see the link for additional information. FDA: [Development & Approval Process \(Drugs\)](#)
  - a. **Single Patient Expanded Access IND** is for an individual patient. There must be FDA and IRB approval prior to treatment. There is no 30-day waiting period before treatment with the investigational product may begin. However, the protocol must be received by the FDA and have IRB approval prior to treatment.
  - b. **Intermediate-Size Patient Population Expanded Access IND** is for more than one patient, but fewer than the number treated under a typical treatment IND protocol for widespread use. The investigational product may or may not be under development for marketing. There is no 30-day waiting period before treatment with the investigational product may begin. However, the protocol must be received by the FDA and have IRB approval prior to treatment.

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c. **Expanded Access for Widespread Use IND** is for a large widespread population, submitted as a protocol under a new IND. The investigational product must be under active development for marketing. Unless the FDA notifies the sponsor the treatment may begin earlier, there is a 30 day waiting period before the treatment may begin.

E. **Open Protocol** - Referred to as **Open Label Protocol, Open Label IND, Continued Access IND or Parallel Track studies**, these uncontrolled studies (no comparator) are typically used when controlled trials have ended and treatment is continued so participants may continue to receive the test article benefits until marketing approval is obtained. These studies involve systematic use of experimental treatments, therefore require the same review and approval as research, including IRB approval and FDA approval in the form of an IDE (medical device) or an IND (drug or biologic).

F. **Off Label Use** - The **Off Label** (FDA approved drug/device being used for a non-approved use) use of a marketed drug or device for individual clinical treatment rather than for research purposes does not require IRB review. However, if the use of the product off label includes data collection and qualifies as human participant research, IRB approval is required. If the goal of the use is to gain FDA approval for a new indication for the test article, an IND or IDE is required and the protocol is subject to IRB review and approval.

G. **Humanitarian Device Exemption** - A **Humanitarian Device Exemption (HDE)** is a special approval given by the FDA to allow marketing of a device designed to treat or diagnose a condition which affects fewer than 4,000 individuals per year. An HDE is granted even though the efficacy of the device has not been tested or proven, because it is not financially feasible to conduct standard clinical testing when so few individuals are affected. The FDA requires IRB approval prior to use of a Humanitarian Use Device (HUD), even though the use is not considered research. See IRB Policy [Humanitarian Device Exemption \(HDE\)](#) for specific procedures.

## VI. REGULATORY REFERENCES:

- 21 CFR 312.2(b)(1) Off Label Use
- 21 CFR 312 Investigational New Drug Application (IND)
- 21 CFR 812 Investigational Device Exemptions (IDE)
- 21 CFR 312 and 316 Expanded Access to Investigational Drugs for Treatment Use

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**VII. ASSOCIATED POLICIES:**

- IRB Policy [Emergency Use of a Test Article](#)
- IRB Policy [Expedited Review of Research](#)
- IRB Policy [IRB Initial Review of Research Protocols](#)
- IRB Policy [Humanitarian Device Exemption \(HDE\)](#)
- IRB Policy [Investigational Device Studies](#)
- IRB Policy [Defining Human Participant Research](#)

**CORPORATE AUTHORITY:**

Beaumont Health (“BH”) as the corporate parent to William Beaumont Hospital, Botsford General Hospital, and Oakwood Healthcare Inc., (“Subsidiary Hospitals”) establishes the standards for all policies related to the clinical, administrative and financial operations of the Subsidiary Hospitals. The Subsidiary Hospitals, which hold all health facility and agency licenses according to Michigan law, are the covered entities and the providers of health care services under the corporate direction of BH. The Subsidiary Hospitals’ workforces are collectively designated as BH workforce throughout BH policies.