SUBJECT: **Experimental and Investigational Services**
INDEX TITLE: **Medical Management**
ORIGINAL DATE: **January 2013**

This policy applies to the following MedStar Health lines of business:
(Check those that apply.)

COMMERCIAL	[ ] HMO	[ ] PPO	[ ] Fully Insured	[ ] Individual Product	[ ] Marketplace (Exchange)	[X] All
GOVERNMENT PROGRAMS	[ ] MA HMO	[ ] MA PPO	[ ] MA C-SNP	[ ] MA D-SNP	[X] MA All	
	[ ] Medicaid					
OTHER	[X] Self-funded/ASO					

I. POLICY

MedStar Health, Inc. identifies certain medical services to be experimental and investigational. These services include drugs, biologics, treatments, surgical and medical procedures, diagnostics, devices, technology, or supplies that do not have established scientific efficacy in the diagnosis and/or management of certain conditions. Experimental and investigational services do not represent standards of medical care and therefore do not meet criteria for medically necessary services. MedStar Health, Inc. will not cover services identified as experimental and investigational.

Coverage for possible exemptions will be considered when it is medically necessary (refer to CRM.015.MH Medical Necessity policy) and covered under the member's benefit plan.

MedStar Health, Inc. recognizes certain investigative medical devices are researched as part of a Food and Drug Administration (FDA) approved clinical trial, but these devices have not been approved for marketing. This policy establishes guidelines for review of devices with "Investigational Device Exemptions" and those approved by the FDA under "Humanitarian Device Exemptions".

Services that are considered experimental and investigational are not covered by the member's individual benefit plan. In the event that the Centers for Medicare & Medicaid Services (CMS) has a Local Coverage Determination (LCD), National Coverage Determination (NCD), or other CMS coverage document in place regarding a procedure, service, or device deemed experimental and investigational

by MedStar Health, Inc., the relevant LCD, NCD, or other CMS coverage document on that procedure, service, or device will be followed for MedStar Health, Inc. Medicare Advantage Members only.

All denials are based on medical necessity and appropriateness as determined by a MedStar Health, Inc. Medical Director (Medical Director).

II. DEFINITIONS

Device – For purposes of this Policy, the definition of a device will be that established by the U.S. Federal Food Drug & Cosmetic Act, Section 201 (h), as follows:

A device is an instrument, apparatus, implement, machine, contrivance, implant, in vitro reagent, or other similar or related article, including a component part, or accessory which is:

1. Recognized in the official National Formulary, or the United States Pharmacopoeia, or any supplement to them;
2. Intended for use in the diagnosis of disease or other conditions, or in the cure, mitigation, treatment, or prevention of disease, in man or other animals; or
3. Intended to affect the structure or any function of the body of man or other animals, and which does not achieve any of its primary intended purposes through chemical action within or on the body of man or other animals and which is not dependent upon being metabolized for the achievement of any of its primary intended purposes.

Experimental and Investigational Services – Medical Services, collectively including drugs, biologics, treatments, surgical and medical procedures, diagnostics, devices, technology, or supplies as identified by the MedStar Health, Inc., which do not have established peer-reviewed scientific efficacy and are not recognized as standards of care in the diagnosis and management of certain conditions. A medical service will be considered experimental and investigational if any of the following criteria apply:

A. There has not been final unrestricted market approval from the FDA or other appropriate government body if applicable, for the specific use requested in the application.
B. Scientific evidence from peer-reviewed medical literature:
    - Is insufficient to evaluate whether or not there is a health benefit;
    - Does not establish a conclusive benefit in health outcomes;
    - Does not demonstrate equal or greater benefit than accepted standard treatment alternatives;
    - Does not conclusively establish the health outcomes benefit from using the medical service; and

- Health outcome benefit is not demonstrated to be obtainable in other investigational or non-investigational settings.

FDA Device Classifications:
Class I Device – Medical devices that are simple in design, manufacture, and have a history of safe use. They have the least amount of regulatory control, are exempt from premarket notification, and may be exempt from the good manufacturing practices regulation (e.g., tongue depressors, arm slings, hand-held surgical instruments).

Class II Device – Medical devices where General Controls* are not sufficient to assure safety and effectiveness and existing methods/standards/guidance documents are available to provide assurances of safety and effectiveness. In addition to compliance with General Controls, Class II devices are required to comply with Special Controls** (e.g., powered wheelchairs, infusion pumps, and surgical drapes).

Class III Device – Medical devices that have the most stringent regulatory controls, for which General Controls and Special Controls are not sufficient to assure safety and effectiveness. A Pre-Market Approval*** submission to the FDA is typically required to allow marketing of a Class III device. Class III devices generally support or sustain human life or prevent significant impairment of health, or they have potential serious risk to the patient (e.g., artificial heart valves, silicone-gel breast implants, implanted cerebral stimulators).

***General Controls –** Class I medical device regulations that include:

- Registration of companies which are required to register under 21 CFR Part 807.20[2], such as manufacturers, distributors, repackagers and relabelers;
- Medical Device Listing with the FDA of devices to be marketed;
- Manufacturing devices in accordance with Good Manufacturing Practices (GMP) in 21 CFR Part 820;
- Labeling devices in accordance with labeling regulations in 21 CFR Part 801 or 809;
- Submission of a premarket notification *[510(k)]* **** before marketing a device unless exempt.

****Special Controls** – Class II medical device regulation including special labeling requirements, mandatory performance standards, international and U.S. postmarket surveillance and FDA medical device specific guidance.

*****Pre-Market Approval (PMA)** – Premarket approval is the required process of scientific review to ensure the safety and effectiveness of Class III devices. Not all Class III devices require a Pre-Market Approval application to be

marketed.

Class III devices which require premarket approval are those:

- Regulated as new drugs prior to May 28, 1976, also called transitional devices;
- Devices found not substantially equivalent to devices marketed prior to May 28, 1976;
- Class III preamendment devices which, by regulation in 21 CFR, require a premarket approval application.

******510 (k) –** A premarket notification process that allows marketing of Class III devices which are equivalent to devices legally marketed before May 28, 1976, until the FDA has published a requirement for manufacturers of that generic type of device to submit premarket approval data.

Humanitarian Use Device (HUD) – A device that is intended to benefit patients with a disease or condition that is manifested in fewer than 4000 individuals in the US per year. HUDs are exempt from the PMA process. Authorization for marketing of a HUD is obtained from the FDA by application for a Humanitarian Device Exemption (HDE).

Humanitarian Device Exemption (HDE) – An HDE is an exemption that authorizes marketing of a HUD, subject to certain profit and use restrictions defined in Sections 514 and 515 of the Food, Drug and Cosmetic Act. Specifically, it restricts HUDs from being sold for profit, except in narrow circumstances, and allows them only to be used following IRB approval for use in a specific facility, except in certain emergencies. The HDE application is not required to contain results from scientifically valid clinical investigations, but must contain sufficient information to indicate that the device does not pose an unreasonable or significant risk of illness or injury and that its probable benefit outweighs the risk from its use. Additionally, the HDE application must demonstrate that no comparable devices are available to treat or diagnose the disease or condition.

Investigational Device Exemption (IDE) – An exemption approved by an institutional review board (IRB) and/or the FDA (if a significant risk device) that allows an investigational device to be used in a clinical study in order to collect safety and effectiveness data usually required to support a PMA and very occasionally a Premarket Notification (510K). These also include clinical evaluation of certain modifications or newly intended uses of legally marketed devices. All such clinical evaluations of investigational devices, unless exempt, must have an approved IDE before the study is initiated.

For purposes of assisting CMS coverage determinations, the FDA places all approved IDEs in one of two categories:

- Category A – Experimental, defined as:
  - Novel, first-of-a-kind technologies;
  - Innovative devices for which the absolute risk of the device type has not been established and initial questions of safety and effectiveness have not been resolved;
  - The FDA is unsure whether these device types can be safe and effective.

- Category B – Non-Experimental/Investigational, defined as:
  - Types of devices that are newer generations of proven technologies;
  - Initial questions of safety and effectiveness of these devices have been resolved;
  - Device considered to be an evolutionary change from proven technologies.

Medical Services – Drugs, biologics, treatments, surgical and medical procedures, diagnostics, devices, technology, and supplies for prevention, diagnosis, and/or treatment of health care conditions.

III. PURPOSE

The purpose of this policy is to provide the rationale and define the review process for identifying experimental and investigational medical services that are generally not covered by the MedStar Health, Inc.

IV. SCOPE

This policy applies to various MedStar Health, Inc. departments as indicated by the Benefit and Reimbursement Committee. These include but are not limited to Medical Management, Benefit Configuration and Claims Departments.

V. PROCEDURE

A. Medical Description / Background

There is a constant stream of new health care information, developments and applications for medical services. Many of these new developments lack sufficient information from clinical trials to demonstrate clinical safety and/or efficacy and may not provide long-term benefits in health outcomes.

The Patient Safety and Technology Assessment Committee (TAC) and Medical

Management staff of the MedStar Health, Inc. routinely conduct evidence-based reviews of new and emerging medical services. This assessment includes:

- A thorough review of available scientific information, which may include peer-reviewed literature, results of clinical trials, outcomes data, regulatory requirements, and input from professionals in the field of the medical service under review;
- Discussion among a multidisciplinary group of health care providers to achieve an adequate understanding of the medical science and its application;
- An appropriate coverage recommendation based on the sum of the evidence;
- Identification of medical services as "experimental and investigational " according to the definition provided in this Policy.

Services determined to be experimental and investigational are listed and experimental and investigational services which demonstrate a significant body of scientific evidence supporting safety and effectiveness are removed from the list. This list of experimental and investigational services which is updated regularly should not be considered all-inclusive due to the fact that new medical services are being developed/researched every day.

B. Specific Indications

For consideration of coverage for any new medical services or new applications of existing medical services, all of the following must apply:

- The medical service or application must be supported by a significant body of scientific evidence supporting safe and effective long-term outcomes resulting in the same or greater health benefits than established alternatives;
- It must be approved by appropriate regulatory agencies (e.g., FDA) for the specific intended use or purpose;
- The scientific evidence and/or clinical outcomes for the medical service and/or application are peer-reviewed and must be attainable outside the investigational setting;
- Application of the medical service must be within accepted standards of good medical practice; and
- The medical service and/or its application must be appropriate in the treatment of the diagnosis or condition specified in the request.

For devices with IDE exemptions:

- **Category A devices will not be** covered because they are considered experimental and investigational, and therefore not considered reasonable and necessary medical services. Routine care costs of patients participating in clinical trials may be covered if allowed under member's

specific benefit plan by MedStar Health, Inc. (according to MP.078.MH-Clinical Trials - Coverage of Routine Care Costs) upon determination that the device is intended for the diagnosis, monitoring or treatment of an immediately life-threatening disease/condition, but the device itself will not be covered.

- **Category B devices may be** considered for coverage if allowed under member's specific benefit plan and all of the following apply:
    - The device must be used within the context of the FDA approved clinical trial.
    - The device must be used according to the clinical trial's approved patient protocol.
    - The medical necessity of the device must be established for the particular member and medical appropriateness established for the amount, duration, and frequency of use or applications of the service.
    - The setting where the service is furnished must be appropriate according to the member's medical needs, condition, and benefit plan.

For devices with Humanitarian Device Exemptions (HDE):

If appropriate under the member's specific plan, all of the follow must apply for consideration of coverage for a HUD on the basis of a HDE:

- A HUD may only be used in facilities that have an established local Institutional Review Board (IRB) to supervise the clinical testing of the device or service);
- IRB approval for use of the HUD must be current according to the IRB requirements (e.g., updated annually);
- The HUD must only be used for HDE approved indications specified in the product labeling.

C. Limitations

The following limitations are applicable for **Category B** devices:
Category B devices will not be covered if any of the following apply:

- When the services or technologies are in the developmental or testing stage;
- When there is no final regulatory or governmental approval;
- When IDEs are applied in the inpatient setting, where they will be included in the Diagnosis Related Group (DRG) payment.

D. Information Required for Review

All of the following information is necessary and must be provided before a coverage determination will be made:

- Member's age and clinical history;
- Documentation of diagnosis and treatment history;
- For IDEs:
    - Clinical trial/research study name, research study sponsor, device name, and numeric registry number (IDE number);
    - Clinical trial/research study protocol;
    - Current IRB approval letter;
    - Copy of the FDA approval with the scope of the approved indication, if applicable;
    - Manufacturer's invoice for the IDE showing the charge for the device and the cost of the device to the researcher/facility (the insurance charge for the device should not exceed the cost to the researcher/facility);
    - The **Fiscal Review Form** indicating the name of the device, the sponsor, and the line item list of services paid by the sponsor of the clinical trial/research study.
- For HUDs:
    - Copy of the FDA Humanitarian Device approval that includes the registry number and the approval indication(s);
    - Current IRB approval letter;
    - An invoice from the manufacturer of the HUD showing the charge for the device and the cost of the device to the researcher/facility (the insurance charge for the device should not exceed the cost to the researcher/facility).

E. Review Process

1. The Medical Management Ancillary Service staff reviews the request. If the case does not meet the established criteria, it is referred to a MedStar Health, Inc. Medical Director (Medical Director).
2. If referred, the Medical Director determines if the requested service is medically necessary and appropriate.
3. The Medical Management Ancillary Service staff completes the review process and communicates the review decision according to the Timeliness of UM Decisions policy for the member's benefit plan.

F. Documentation Required for Claims Payment

- **ICD-9 Code V70.7 or ICD-10 Code Z00.6** must be reported as the secondary diagnosis
- Utilization of appropriate modifiers Q0 and/or Q1

G. Variations

Generally, the CHIP and Medical Assistance products do not cover IRB studies using IDE/HUDs as these are considered research and experimental/investigative services.

For Commercial Self-Funded (ASO) groups:
The applicability of this policy to individuals in self-funded commercial groups is subject to the contractually agreed upon Schedule of Benefits associated with the specific coverage/plan design that each self-funded group has purchased from any entity within the UPMC Insurance Services Division. Whether coverage/payment for the services and/or benefits governed by this policy are available to a self-funded group member is determined on a case by case basis, based on the benefit plan of that member's Schedule of Benefits and, unless expressly stated otherwise, any authorization/medical necessity requirements described herein.

H. Records Retention

Records Retention for documents, regardless of medium, are provided within the MedStar Health, Inc., and as indicated in the MedStar Health, Inc. Policy and Procedure CORP.028.MH Records Retention.

Unless otherwise mandated by Federal or State law, or unless required to be maintained for litigation purposes, any communications recorded pursuant to this Policy are maintained for a minimum of ten (10) years from the date of recording.

I. References

Medical LiteratureClinical Information

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12. Social Security Act. Section 1862 (42 USC 1395y). Exclusions from Medicare Coverage and Medicare as Secondary Payer. http://www.ssa.gov/OP_Home/ssact/title18/1862.htm
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Disclaimer:

MedStar Health Inc. medical payment and prior authorization policies do not constitute medical advice and are not intended to govern or otherwise influence the practice of medicine. The policies constitute only the reimbursement and coverage guidelines of MedStar Health Inc. and its affiliated managed care entities. Coverage for services varies for individual members in accordance with the terms and conditions of applicable Certificates of Coverage, Summary Plan Descriptions, or contracts with governing regulatory agencies.

MedStar Health Inc. reserves the right to review and update the medical payment and prior authorization guidelines in its sole discretion. Notice of such changes, if necessary, shall be provided in accordance with the terms and conditions of provider agreements and any applicable laws or regulations.

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