Experimental or Investigational Treatment Utilization Review Decisions

Policy Number: **M20150623055**

Effective Date: **1/1/1984**

Sponsoring Department: **Health Care Services**

Impacted Department(s): **Health Care Services**

Type of Policy: ☐ Internal ☒ External

Data Classification: ☐Confidential ☐Restricted ☒Public

Applies to (Line of Business):

☐ Corporate (All)

☒ State Products, if yes which plan(s): ☒MediSource; ☒MediSource Connect; ☒Child Health Plus; ☒Essential Plan

☒ Medicare, if yes, which plan(s): ☒MAPD; ☐PDP; ☒ISNP; ☒CSNP

☒ Commercial, if yes, which type: ☒Large Group; ☒Small Group; ☒Individual

☒ Self-Funded Services *(Refer to specific Summary Plan Descriptions (SPDs) to determine any pre-authorization or pre-certification requirements and coverage limitations. In the event of any conflict between this policy and the SPD of a Self-Funded Plan, the SPD shall supersede the policy.)*

Excluded Products within the Selected Lines of Business (LOB)

N/A

Applicable to Vendors? Yes ☒ No☐

Purpose and Applicability:

To render decisions regarding coverage for medical, behavioral, surgical or pharmacological treatments, testing and interventions which are not scientifically proven to be safe, efficacious, and/or demonstrate clinical utility.

Policy:

Commercial, Self-Funded, Medicare Advantage, MediSource, MediSource Connect, Essential Plan and Child Health Plus:

Authorization for an **Experimental** or **Investigational** or **Unproven** treatment may be considered if it is determined, based on **medical and scientific evidence** and that such a treatment could positively affect the member's health status and where commonly accepted medical procedures have either proven unsuccessful or are known to be harmful or ineffective to the individual member.

Regulatory approval, from an entity such as the United States Food and Drug Administration (FDA), will be considered in determining if a service is experimental, investigational or unproven. Regulatory approval does not necessarily mean that the service has a proven benefit, or that it is an appropriate or an effective treatment for a specific diagnosis or specific medical disorder.

In determining whether a service is an experimental, investigational or unproven treatment that will be covered, **all** of the following criteria must be met:

1. A requested service, that is a medical device, drug or biological product must have received final approval from appropriate regulatory agencies (i.e., the FDA). Any approval given as an interim step in the FDA regulatory process such an Investigational Device Exemption is not automatically sufficient.

2. Published peer-reviewed medical literature must provide conclusive evidence that:
    a. The service has a defined, positive effect on health outcomes from well-designed studies with positive endorsements from national medical professional organizations or boards regarding scientific efficacy and rationale; and

    b. The service leads to improvements in health outcomes with the beneficial effects of the service outweighing any harmful effects; and

    c. The service is at least as effective in improving health outcomes as established services or technologies or is usable in appropriate clinical contexts in which an established service or technology is not employable; and

    d. The improvement in health outcomes is possible in standard conditions of medical practice, outside clinical investigatory settings.

Requests will be denied if they are not currently in an approved study, or not within the current accepted standards of medical care.

All requests regarding coverage for interventions considered to be investigational, experimental or unproven will be reviewed for determination by an Independent Health medical director on an individual case basis.

An evaluation of the peer-reviewed scientific literature, including but not limited to subscription materials, has provided Independent Health the basis for its medical necessity coverage outlined above.

Relevant information, including but not limited to, the following sources, may be utilized and considered in the decision-making process:

- Current scientific literature,
- Experts consulted,
- Hayes Inc. Knowledge Center,
- UpToDate®,
- Professional organizations and societies including, but not limited to, the American College of Obstetrics and Gynecologists (ACOG), American Society of Clinical Oncologists (ASCO), and the National Comprehensive Cancer Network (NCCN),
- Clinical Practice Guidelines of the US Department of Health and Human Services,
- Health Technology Assessment of the US Department of Human Health and Services,
- Directives and Bulletins from the New York State Department of Health, and
- The Department of Financial Services.

Clinical Trials
Experimental or Investigational Treatment that is part of a clinical trial will be considered according to the Clinical Trials policy. See separate policy listed in the **Reference** section of this policy.

Pre-Authorization Required? Yes ☒ No☐

Pre-authorization is required for all experimental, investigational or unproven treatments.

Definitions

Experimental Services - Not approved by the U.S. Food and Drug Administration (FDA) to be lawfully marketed for the proposed use, or not identified in the American Hospital Formulary Service as appropriate for the proposed use.

Investigational Services - The subject of an ongoing clinical trial that meets the definition of a Phase I, II, or III clinical trial set forth in the FDA regulation, regardless of whether the trial is subject to FDA oversight

Medical and Scientific Evidence means reports of well-designed clinical trials with measurable results, which have been critically reviewed for scientific accuracy, validity, and reliability by unbiased independent experts including peer-reviewed scientific journals and national medical organizations.

Unproven Services - services which regardless of approval by the appropriate governmental regulatory body including but not limited to the U.S. Food and Drug Administration, CLIA, etc., are:

- Not consistent with conclusions of published, peer-reviewed literature that demonstrates that the service has an equivalent or superior beneficial effect on health outcomes when compared to established treatments or technologies; and
- Equivalent or superior safety profile when compared to established treatments or technologies; or
- Not based on trials that meet either of the following designs:

- Well-conducted randomized placebo-controlled trials. (Two or more treatments are compared to each other, and the patient is randomly assigned to one treatment or the other); or
- Well-conducted cohort studies. (Patients who receive the study treatment are compared to a group of patients who receive standard therapy. The comparison group must be nearly identical to the study treatment group.); and
- The sample size of the study is of sufficient power to allow calculation of statistically significant results, and from which one can draw clinically significant conclusions.

References

Related Policies, Processes and Other Documents
Clinical Trials, Policy No.: M011011296

Non-Regulatory references
Independent Health, Group Health Contract; Section 5: Limitations of Coverage, Exclusions and Services Not Covered.

Regulatory References
10 New York Codes, Rules and Regulations Part 98.

Medicaid Managed Care/Family Health Plus/HIV Special Needs Plan Model Contract, March 1, 2019, Appendix K, Section 36.

This policy contains medical necessity criteria that apply for this service. Please note that payment for covered services is subject to eligibility criteria, contract exclusions and the limitations noted in the member's contract at the time the services are rendered.

Version Control

Signature / Approval on File? Yes ☒ No☐

Revision Date	Owner	Notes
1/1/2024	Health Care Services	Revised
10/1/2023	Health Care Services	Revised
10/1/2022	Health Care Services	Reviewed
11/1/2021	Health Care Services	Revised
10/1/2020	Health Care Services	Reviewed
11/1/2019	Medical Management	Revised
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10/1/2016	Medical Management	Revised
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4/1/2014	Medical Management	Revised
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10/19/2001	Medical Management	Reviewed
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