Geisinger

Geisinger Health Plan Policies and Procedure Manual

Policy: MP333

Section: Medical Benefit Policy

Subject: Coverage for Treatment of Rare Disease

Applicable Lines of Business

Commercial	**X**	**CHIP**	
Medicare	**X**	**ACA**	**X**
Medicaid			

I. Policy: Coverage for Treatment of Rare Disease

II. Purpose/Objective:

To provide a policy of coverage regarding coverage for treatment of rare disease

III. Responsibility:

A. Medical Directors
B. Medical Management

IV. Required Definitions

1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than the department requiring/authoring the policy.
3. Devised – the date the policy was implemented.
4. Revised – the date of every revision to the policy, including typographical and grammatical changes.
5. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions

Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
b. provided for the diagnosis, and the direct care and treatment of the Member's condition, illness disease or injury;
c. in accordance with current standards of good medical treatment practiced by the general medical community.
d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient.

Medicaid Business Segment

Medically Necessary — A service, item, procedure, or level of care that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

- Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
- Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
- Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age

Center of Excellence: a facility or entity that provides leadership, best practices, research, support and/or training for a focus area. Academic medical centers that routinely conduct nationally sponsored, multi-centered clinical trials in the category of disease the member has, and have received designation as an institution with special standing in the treatment of the disease (for example, a Comprehensive Cancer Center for the treatment of cancer).

Unproven services or therapies are treatments or procedures that lack sufficient medical documentation and/or evidence to support their clinical effectiveness and not generally recognized as effective or appropriate for the treatment of the member's particular condition or disease

THIS POLICY DOES NOT APPPLY TO THE MEDICAID BUSINESS SEGMENT

DESCRIPTION:
The purpose of this policy is to outline the process followed by Geisinger Health Plan (hereafter referred to as the Plan) in determining coverage decisions related to the diagnosis and treatment of rare disease. According to the National Institutes of Health, a disease is considered rare if it has a prevalence of fewer than 200,000 affected individuals in the United States. There are roughly 7,000 rare diseases currently identified for which established standards of care, or published peer-reviewed medical literature regarding outcomes or comparative effectiveness of treatment options may be limited or absent.

PROCESS:
A Plan Medical Director will establish and consider all of the following information and at his/her discretion determine whether the requested treatment or therapy is clinically reasonable and medically necessary to treat the rare disease.

- **Confirmation of diagnosis** – the diagnosis has been established beyond reasonable doubt and documented in the medical record based on physical examination, laboratory and/or molecular testing (if applicable) results, imaging studies, and other applicable standard diagnostic tests.

- **Requesting provider's experience in treating the rare disease** – the medical director will attempt to establish the requesting provider's experience in diagnosing and treating persons with the identified rare disease. At a minimum, the requesting provider should be a board certified or board eligible physician qualified to practice in the area of practice appropriate to treat the member's condition.

- **Review of published evidence*** – the requested service or therapy is supported in the currently available published medical literature and is felt to be clinically reasonable to treat the member's rare disease based on results of clinical trials or articles published in the peer reviewed medical literature showing that the proposed treatment is likely to benefit members who have the specific rare disease.

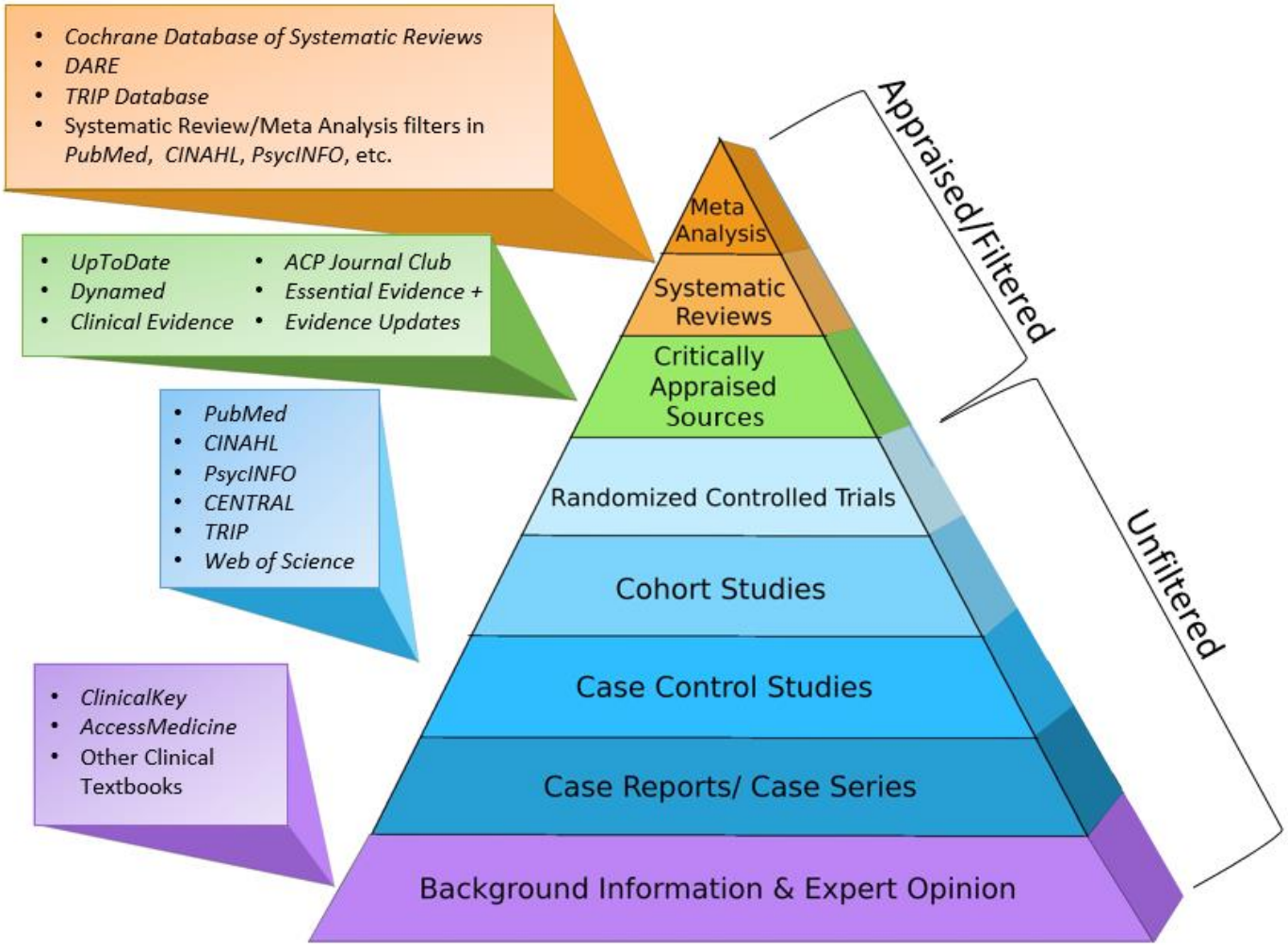
- **Expert consultation** – if possible, the medical director will seek opinion of an independent specialist or consultant with expertise in the area of practice appropriate to treat the member's rare disease, preferably at an established and recognized Center of Excellence for the condition. If the requesting provider has experience in treating the rare disease, and there is disagreement between the requesting provider and the consultant, a second expert opinion shall be sought if possible. If a second independent expert opinion cannot be obtained, the medical director will make the determination based on available evidence.

- **Review of available treatment options** – the requesting provider has considered all of the available treatment options and based on the consultant's opinion, the benefits of the requested treatment are likely to outweigh the potential risks. If the requested service or therapy is not considered to be a standard treatment, and standard treatments are available, the requesting provider must establish why the standard therapy available would not be beneficial, would be ineffective or inappropriate.

- **Clinical trials** – If the requested service or therapy is not considered to be a standard treatment of the disease, the medical director will establish whether the requested service or therapy is available to the member through an

actively recruiting clinical trial. Coverage for routine and customary services while participating in an approved clinical trial is outlined in **MP312 Routine Care in Clinical Trials**

***EVIDENCE REVIEW**
The highest available level of evidence will be preferentially used to guide determinations of coverage.



New York Medical College: EBM Resource Center: Acquire the Evidence

Appraised/Filtered resources - evaluated for quality and often contain recommendations for practice.
Unfiltered resources - primary or original research studies

LIMITATIONS:
Coverage is subject to the member's benefits and exclusions outlined in their specific program benefit document.

Unproven services or therapies are treatments or procedures that lack sufficient medical documentation and/or evidence to support their clinical effectiveness and not generally recognized as effective or appropriate for the treatment of the member's particular condition or disease.

EXCLUSIONS:
The Plan does NOT provide coverage for the use of experimental, investigational or unproven services or therapies. There is insufficient evidence in the peer-reviewed published medical literature to establish the effectiveness of these modalities on health outcomes when compared to established tests or technologies**.**

Medicaid Business Segment:
Any requests for services, that do not meet criteria set in the PARP, may be evaluated on a case by case basis.

Note: A complete description of the process by which a given technology or service is evaluated and determined to be experimental, investigational or unproven is outlined in MP 15 - Experimental Investigational or Unproven Services or Treatment.

CODING ASSOCIATED WITH: Rare Disease
The following codes are included below for informational purposes and may not be all inclusive. Inclusion of a procedure or device code(s) does not constitute or imply coverage nor does it imply or guarantee provider reimbursement. Coverage is determined by the member specific benefit plan document and any applicable laws regarding coverage of specific services. Please note that per Medicare coverage rules, only specific CPT/HCPCS Codes may be covered for the Medicare Business Segment. Please consult the CMS website at www.cms.gov or the local Medicare Administrative Carrier (MAC) for more information on Medicare coverage and coding requirements.

0212U Rare diseases (constitutional/heritable disorders), whole genome and mitochondrial DNA sequence analysis, including small sequence changes, deletions, duplications, short tandem repeat gene expansions, and variants in non-uniquely mappable regions, blood or saliva, identification and categorization of genetic variants, proband (Do not report 0212U in conjunction with 81425) Genomic Unity® Exome Plus Analysis -proband
0213U Rare diseases (constitutional/heritable disorders), whole genome and mitochondrial DNA sequence analysis, including small sequence changes, deletions, duplications, short tandem repeat gene expansions, and variants in non-uniquely mappable regions, blood or saliva, identification and categorization of genetic variants, each comparator genome (eg, parent, sibling) (Do not report 0213U in conjunction with 81426) Genomic Unity® Exome Plus Analysis - comparator
0214U Rare diseases (constitutional/heritable disorders), whole exome and mitochondrial DNA sequence analysis, including small sequence changes, deletions, duplications, short tandem repeat gene expansions, and variants in non-uniquely mappable regions, blood or saliva, identification and categorization of genetic variants, proband (Do not report 0214U in conjunction with 81415) Genomic Unity® Exome Plus Analysis-proband
0215U Rare diseases (constitutional/heritable disorders), whole exome and mitochondrial DNA sequence analysis, including small sequence changes, deletions, duplications, short tandem repeat gene expansions, and variants in non-uniquely mappable regions, blood or saliva, identification and categorization of genetic variants, each comparator exome (eg, parent, sibling) (Do not report 0215U in conjunction with 81416) Genomic Unity® Exome
0335U Rare diseases (constitutional/heritable disorders), whole genome sequence analysis, including small sequence changes, copy number variants, deletions, duplications, mobile element insertions, uniparental disomy (UPD), inversions, aneuploidy, mitochondrial genome sequence analysis with heteroplasmy and large deletions, short tandem repeat (STR) gene expansions, fetal sample, identification and categorization of genetic variants (IriSight™ Prenatal Analysis)
0336U Rare diseases (constitutional/heritable disorders), whole genome sequence analysis, including small sequence changes, copy number variants, deletions, duplications, mobile element insertions, uniparental disomy (UPD), inversions, aneuploidy, mitochondrial genome sequence analysis with heteroplasmy and large deletions, short tandem repeat (STR) gene expansions, blood or saliva, identification and categorization of genetic variants, each comparator genome (eg, parent) (IriSight™ Prenatal Analysis)

LINE OF BUSINESS:
Eligibility and contract specific benefits, limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy. For Medicare, applicable LCD's and NCD's will supercede this policy.

REFERENCES:
National Institute for Health and Care Excellence Guides to the methods of technology appraisal 2013. National Institute for Health and Care Excellence website. http://www.nice.org.uk/article/pmg9/chapter/foreword. Published April 2013

Teutsch S. Systems for Research and Evaluation for Translating Genome-Based Discoveries for Health: Workshop Summary. Generating Evidence for Decision Making. Does the Type of Decision Being Made Influence the Evidence Needed? https://www.ncbi.nlm.nih.gov/books/NBK32309/

Institute for Clinical and Economic Review (ICER).
Real World Evidence for Coverage Decisions: Opportunities and Challenges.A Report from the 2017 ICER Membership Policy Summit. March 2018 https://icer-review.org/wp-content/uploads/2018/03/ICER-Real-World-Evidence-White-Paper-03282018.pdf

This policy will be revised as necessary and reviewed no less than annually.

Devised: 12/19

Revised:

Reviewed: 12/20, 12/21, 12/22

Geisinger Health Plan may refer collectively to health care coverage sponsors Geisinger Health Plan, Geisinger Quality Options, Inc., and Geisinger Indemnity Insurance Company, unless otherwise noted. Geisinger Health Plan is part of Geisinger, an integrated health care delivery and coverage organization.

Coverage for experimental or investigational treatments, services and procedures is specifically excluded under the member's certificate with Geisinger Health Plan. Unproven services outside of an approved clinical trial are also specifically excluded under the member's certificate with Geisinger Health Plan. This policy does not expand coverage to services or items specifically excluded from coverage in the member's certificate with Geisinger Health Plan. Additional information can be found in MP015 Experimental, Investigational or Unproven Services.

Prior authorization and/or pre-certification requirements for services or items may apply. Pre-certification lists may be found in the member's contract specific benefit document. Prior authorization requirements can be found at https://www.geisinger.org/health-plan/providers/ghp-clinical-policies