**MVP Health Care Medical Policy**

**Medicare Part B: Orphan Drug(s) and Biologicals**

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| **Type of Policy:** | **Drug/Medical Therapy** |
| **Prior Approval Date:** | **08/01/2024** |
| **Approval Date:** | **07/01/2025** |
| **Effective Date:** | **09/01/2025** |

**Related Policies:** **Experimental or Investigational Procedures, Behavioral Health Services, Drugs & Treatments, Off-Label use of FDA Approved Drugs, Clinical Trials**

**Refer to relevant CMS LCDs/NCDs/Policy Articles for most up to date Medicare Part B guidance if available**

***Codes Requiring Prior Authorization (covered under the medical benefit)**

Adagen® (J2504 Inj, pegademase bovine, 25 IU)
Adzynma (J7171, ADAMTS13, Recombinant-krhn)
Aldurazyme® (J1931 Inj, laronidase, 0.1 mg)
Arikayce (J8499 amikacin liposome inhalation susp)
Brineura (J0567 cerliponase alfa inj. 1mg)
Cablivi (J3590, Inj Caplacizumab)
Ceprotin™ (J2724 Inj, protein C concentrate, IV 101U)
Clolar® (J9027 Inj, clofarabine, 1 mg)
Crysvita (J0584 inj, burosumab-twza 1 mg)
Elaprase® (J1743 Inj, idursulfase)
Elzonris (J9269 Inj, tagraxofusp-erzs)
Enjaymo™ (J1302 Inj, sutimlimab-jome)
Evkeeza™(J1305, evinacumab-dgnb)
Folotyn (J9307 Inj, pralatrexate, 1 mg)
Fusilev™ (J0641 Inj, levoleucovorin 0.5mg)
Gamifant (J9210, emapalumab-IZSG inj)
Ilaris® (J0638 Inj, canakinumab 1mg)
Imaavy (nipocalimab-aahu IV)

Kanuma (J2840 Inj, sebelipase alfa, 1mg)
Khapzory (J0642) levoleucovorin IV solution)
Lamzede (J0217, velmanase alfa-tycv, 10mg)
Lumizyme (J0221 Inj, alglucosidase alfa)
Mepsevii (J3397 Inj. Vestronidase alfa)
Naglazyme® (J1458 Inj, galsulfase, 1 mg)
Nexviazyme® (J0219, avalglucosidase alfa-ngpt)
Nulibry® (C9399, J3490, fosdenopterin)
Oxlumo® (J0224, lumasiran)
Piasky (J1307, crovalimab-akkz)
Pombiliti (J1203, cipaglucosidase alfa, powder for injection)
Poteligeo (J9204) Inj. mogamulizumab-kpkc)
Reblozyl (J0896, luspatercept-aamt, SQ injection)
Retisert® (J7311 fluocinolone acetonide, intravitreal implant)
Rivfloza Vials (C9399, J3490, nedosiran)
Rystiggo (J9333, Rozanolixizumab SQ infusion)
Scenesse (J7352, afamelanotide implant 16mg)
Sylvant (J2860 Inj, siltuximab, 10mg)
Uplizna® (J1823, inebilizumab-cdon)
Veopoz (J9376, pozelimab-bbfg, injection, 1mg)
Vimizim (J1322, Inj, elosulfase alfa, 1mg)
Vyjuvek (J3401, beremagene geperpavec)
Vyvgart™ (J9332 Inj, efgartigimod alfa-fcab)
Vyvgart Hytrulo (J9334 Injection, efgartigimod alfa; hyaluronidase)
Xenpozyme™ (J0218 Inj, olipudase alfa)
Zynlonta (J9359, loncastuximab tesirine-lpyl)
** This list is subject to change based on FDA approval of new drugs and/or new indications*

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Refer to the MVP website for the Medicare Part D formulary and policies for drugs that may covered under the Part D benefit.

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**Overview/Summary of Evidence**

An orphan drug is a drug used to treat a rare disease or condition which affects:

- less than 200,000 persons in the United States[1]; or
- more than 200,000 persons in the United States; and there is no reasonable expectation that the cost of developing and making a drug will be recovered from sales in the United States[1].

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**Indications/Criteria**

Orphan drugs or FDA approved drugs designated with an orphan drug indication may be covered on a case-by-case basis, with prior authorization, for the FDA approved indications only. Only drugs FDA approved for marketing as Orphan Drugs or Biologics will be considered for coverage under this policy.

The drug must be prescribed by a plan affiliated Specialist familiar with the treatment of the rare disease or condition.

Those drugs listed at https://www.accessdata.fda.gov/scripts/opdlisting/oopd/ have been designated by the FDA as Orphan Designated Products approved for marketing. The list is maintained by the FDA and is subject to change.

Physician and member must comply with all approved and/or limited distribution channels for the agent including specialty pharmacy vendors where applicable.

Drug and/or biological coverage is subject to the terms and conditions of the member's prescription drug rider and/or contract.

Documentation submitted must include baseline subjective/objective laboratory or test results (dependent on drug and diagnosis). If member started therapy while enrolled in a clinical trial, baseline laboratory or test results must be provided from prior to the start of the trial.

For continuation of therapy request documentation must show improvement in symptoms/condition from baseline.

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**Exclusions**

The use of orphan drugs and biologics will not be considered medically necessary for the following situations:

- Age, dose, frequency of dosing, and/or duration of therapy outside of FDA approved package labeling Member has not failed all other standard therapies for the disease

- FDA warnings and contraindications for the use of the drug have not been addressed by the prescriber

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## References


1. U.S. Food and Drug Administration (FDA). Orphan Drug Act Congressional Findings for the Orphan Drug Act. Available: http://www.fda.gov/regulatoryinformation/legislation/federalfooddrugandcosmeticactfdcact/significantamendmentstothefdcact/orphandrugact/default.htm
2. U.S. Food and Drug Administration (FDA). Developing Products for Rare Diseases & Conditions. Available: http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm
3. U.S. Food and Drug Administration (FDA). FDA Application. Search Orphan Drug Designations and Approvals [Database]. Available: http://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm.