

# RARE CONDITIONS PA POLICY



**Included Products:** Therapies on medical and pharmacy benefits

*Created: 01/10/2019      Revised: 11/14/2024      Reviewed: 11/14/2024      Updated: 12/01/2024*

*Italicized are nonformulary for outpatient benefit. PA required on medical benefit.*

*Due to the rare conditions these drugs are used for, all reviews are subject to an evaluation of medical necessity/appropriateness by a CareOregon Medical Director and/or outside specialist reviewer.*

| Brand             | Generic                           |
|-------------------|-----------------------------------|
| <i>Adzynma</i>    | <i>ADAMTS13, recombinant-krhn</i> |
| <i>Scenesse</i>   | <i>afamelanotide</i>              |
| <i>Tecelra</i>    | <i>afamitresgene autoleucel</i>   |
| <i>Fabrazyme</i>  | <i>agalsidase beta</i>            |
| <i>Ceredase</i>   | <i>alglucerase</i>                |
| <i>Lumizyme</i>   | <i>alglucosidase alfa</i>         |
| <i>Myozyme</i>    | <i>alglucosidase alfa</i>         |
| <i>Vijoice</i>    | <i>alpelisib</i>                  |
| <i>Nexviazyme</i> | <i>avalglucosidase alfa-ngpt</i>  |
| <i>Nuloxix</i>    | <i>belatacept</i>                 |
| <i>Vyjuvek</i>    | <i>beremagene geperpavec-svdt</i> |
| <i>Zynteglo</i>   | <i>betibeglogene autotemcel</i>   |
| <i>Ilaris</i>     | <i>canakinumab</i>                |
| <i>Amondys 45</i> | <i>casimersen</i>                 |
| <i>Oxervate</i>   | <i>cenergermin-bk bj</i>          |
| <i>Brineura</i>   | <i>cerliponase alfa</i>           |

|                   |  |
|-------------------|--|
| <i>Pombiliti</i>  | <i>cipaglucosidase alfa-atga</i>         |
| <i>Acthar</i>     | <i>corticotropin inj gel</i>             |
| <i>Cortrophin</i> | <i>corticotropin inj gel</i>             |
| <i>Defitelio</i>  | <i>defibrotide</i>                       |
| <i>Elevidys</i>   | <i>delandistrogene moxeparvovec-rokl</i> |
| <i>Igalmi</i>     | <i>dexmedetomidine</i>                   |
| <i>Revcovi</i>    | <i>elapegademase-lvrl</i>                |
| <i>Skysona</i>    | <i>elivaldogene autotemcel</i>           |
| <i>Iqirvo</i>     | <i>elafibrinor</i>                       |
| <i>Vimizim</i>    | <i>elosulfase alfa</i>                   |
| <i>Gamifant</i>   | <i>emapalumab-lzsg</i>                   |
| <i>Exondys 51</i> | <i>eteplirsen</i>                        |
| <i>Hemgenix</i>   | <i>etranacogene dezaparvovec-drlb</i>    |
| <i>Evkeeza</i>    | <i>evinacumab-dgnb</i>                   |
| <i>Beqvez</i>     | <i>Fidanacogene elaparvovec-dzkt</i>     |
| <i>Nulibry</i>    | <i>fosdenopterin</i>                     |
| <i>Naglazyme</i>  | <i>galsulfase</i>                        |
| <i>Givlaari</i>   | <i>givosiran</i>                         |
| <i>Duvyzat</i>    | <i>givinostat</i>                        |
| <i>Voraxaze</i>   | <i>glucarpidase</i>                      |
| <i>Vyondys 53</i> | <i>golodirsen</i>                        |
| <i>Elaprerase</i> | <i>idursulfase</i>                       |
| <i>Cerezyme</i>   | <i>imiglucerase</i>                      |
| <i>Alferon-N</i>  | <i>Interferon alfa-n3</i>                |
| <i>Aldurazyme</i> | <i>laronidase</i>                        |

|                  |   |
|------------------|---|
| <b>Joenja</b>    | leniolisib                                  |
| <b>Zokinvy</b>   | lonafarnib                                  |
| <b>Oxlumo</b>    | <i>lumasiran</i>                            |
| <b>Livmarli</b>  | maralixibat                                 |
| <b>Pyrukynd</b>  | mitapivat                                   |
| <b>Danyelza</b>  | <i>naxitamab-gqqk</i>                       |
| <b>Spinraza</b>  | <i>nusinersin</i>                           |
| <b>Ocaliva</b>   | <i>Obeticholic acid</i>                     |
| <b>Xenpozyme</b> | <i>olipudase alfa</i>                       |
| <b>Skyclarys</b> | omaveloxolone                               |
| <b>Zolgensma</b> | <i>onasemnogene abeparvovec</i>             |
| <b>Sohonos</b>   | palovarotene                                |
| <b>Onpattro</b>  | <i>patisiran</i>                            |
| <b>Empaveli</b>  | <i>pegcetacoplan subcutaneous injection</i> |
| <b>Krystexxa</b> | <i>pegloticase</i>                          |
| <b>Elfabrio</b>  | <i>pegunigalsidase alfa-iwxj</i>            |
| <b>Ryplazim</b>  | <i>plasminogen</i>                          |
| <b>Veopoz</b>    | <i>pozelimab-bbfg</i>                       |
| <b>Kanuma</b>    | <i>sebelipase Alfa</i>                      |
| <b>Koselugo</b>  | selumetinib                                 |
| <b>Enjaymo</b>   | <i>sutimlimab-jome</i>                      |
| <b>Vyndamax</b>  | tafamidis                                   |
| <b>Vyndaqel</b>  | tafamidis meglumine                         |
| <b>Elelyso</b>   | <i>taliglucerase</i>                        |
| <b>Dojolvi</b>   | triheptanoin                                |

|                  |  |
|------------------|--|
| <i>Roctavian</i> | <i>valoctocogene roxaparvovec-rvox</i> |
| <i>Vpriv</i>     | <i>velaglucerase alfa</i>              |
| <i>Lamzede</i>   | <i>velmanase alfa-tycv</i>             |
| <i>Mepsevii</i>  | <i>vestronidase alfa-vjbk</i>          |
| <i>Viltepso</i>  | <i>viltolarsin</i>                     |
| <i>Luxturna</i>  | <i>voretigine neparvovec</i>           |
| <i>Voxzogo</i>   | <i>vosoritide</i>                      |
| <i>Amvuttra</i>  | <i>vutrisiran</i>                      |

## All Diagnoses

| Initial Criteria: All Diagnoses |  | If yes          | If no           |
|---------------------------------|--|-----------------|-----------------|
| 1.                              | Is there quality medical evidence to demonstrate the medication will be effective in treating the diagnosis?   | Continue to #2. | Do not approve. |
| 2.                              | Are there any concerns for serious adverse events or contraindications that make using the medication unsafe?  | Do not approve. | Continue to #3. |
| 3.                              | <p>Does the request meet the definition of medical appropriateness per Oregon Administrative Rules as defined by meeting ALL of the following?:</p> <ul style="list-style-type: none"> <li>a. Requested by or in consultation with a licensed health care provider with the appropriate specialty to treat the condition; AND</li> <li>b. Is recognized to be safe, effective, and appropriate by the relevant professional community/guidelines; AND</li> <li>c. Is not solely for the convenience or preference of the member; AND</li> </ul> <p>The most cost-effective option that can be safely and effectively provided.</p> | Continue to #4. | Do not approve. |

|                         |  |                 |                 |
|-------------------------|--|-----------------|-----------------|
| <b>4.</b>               | <p>Does the request meet the definition of medical necessity per Oregon Administrative Rules defined as meeting one or more of the following:</p> <ul style="list-style-type: none"> <li>a. Prevention, diagnosis, or treatment of a disorder that results in health impairment or a disability; OR</li> <li>b. Improve the ability for the member to achieve age-appropriate growth and development; OR</li> </ul> <p>Establish the ability for a member to attain, maintain, or regain independence in self-care, ability to perform activities of daily living, or improve health status.</p> | Continue to #5. | Do not approve. |
| <b>5.</b>               | Approve for up to 12 months.   |                 |                 |
| <b>Renewal Criteria</b> |  | <b>If yes</b>   | <b>If no</b>    |
| <b>1.</b>               | Has there been recent chart note documentation of response and medical necessity to continue treatment?  | Continue to #2. | Do not approve. |
| <b>2.</b>               | Approve for up to 12 months.   |                 |                 |