State Medicaid Drug Review Processes for Rare Disease Treatments

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LET MY DOCTORS DECIDE
Action Network

PREPARED BY LET MY DOCTORS DECIDE ACTION NETWORK

For the more than 70 million Americans enrolled in Medicaid, state governments serve as the primary gatekeepers to prescription drug coverage. This role carries particular weight for patients living with a rare disease, where FDA-approved treatments are often the result of years of scientific advancement and represent a patient's best or only therapeutic option. Each state Medicaid program establishes its own framework for evaluating new drugs, setting coverage policies, and determining when and how stakeholders can weigh in on those decisions. The result is a patchwork of processes that can mean vastly different access outcomes for patients depending solely on the state where the patient lives.

Let My Doctors Decide Action Network (LMDDAN) explored Medicaid coverage processes for four rare disease treatments across nine diverse states: Colorado, Georgia, Illinois, Kansas, Massachusetts, Minnesota, Mississippi, Oregon and Virginia. While some similarities were found, there were also significant differences in the opportunities for prescribing physicians, patients and patient advocates to share their perspectives on how the treatment being reviewed could address unmet health needs, and for drug manufacturers to present relevant clinical and scientific information about treatments under consideration.

There is little uniformity in how states develop coverage policies and their use of Pharmacy & Therapeutics Committees (P&T), Drug Utilization Review (DUR) Boards or similar entities.

Review of four FDA-approved drugs from FDA approval through September 2025:

Skyclarys (omaveloxolone) is indicated for the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older.[1]

Livdelzi (seladelpar) is a peroxisome proliferator-activated receptor (PPAR)-delta agonist indicated for the treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults who have an inadequate response to UDCA, or as monotherapy in patients unable to tolerate UDCA. This indication is approved under accelerated approval based on a reduction of alkaline phosphatase (ALP). Improvement in survival or prevention of liver decompensation events have not been demonstrated. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).[2]

Elevidys (delandistrogene moxeparvovec-rokl) is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients 4 years of age and older with Duchenne muscular dystrophy (DMD) who are ambulatory and have a confirmed mutation in the DMD gene.[3]

Qalsody (tofersen) is an antisense oligonucleotide indicated for the treatment of amyotrophic lateral sclerosis (ALS) in adults who have a mutation in the superoxide dismutase 1 (SOD1) gene. This indication is approved under accelerated approval based on reduction in plasma neurofilament light chain observed in patients treated with QALSODY. Continued approval for this indication may be contingent upon verification of clinical benefit in confirmatory trial(s).[4]

1. https://www.biogencdn.com/us/sky/prescribing-information.pdf
2. https://www.gilead.com/-/media/files/pdfs/medicines/pbc/livdelzi/livdelzi_pi.pdf
3. https://www.elevidys.com/pi
4. https://www.biogencdn.com/us/pdfs/qalsody-prescribing-information.pdf

METHODOLOGY USED TO IDENTIFY THE PROCESS FOR REVIEWING A NEW TREATMENT BY DUR AND P&T COMMITTEES

For the nine states, LMDDAN identified the process used by each state's DUR and P&T Committee (or similar named entity) to review a new FDA-approved drug for a rare disease. Four recent FDA-approved drugs were tracked through each state's review process. The analysis was conducted using information from state Medicaid websites, personal observations of Pharmacy & Therapeutics (P&T) Committee and Drug Utilization Review (DUR) Board meetings, and other public information. The findings illustrate the variation of policies and practices of state Medicaid public drug reviews at any given point of time.

The methodology included collecting the follow information for each state:

- State name
- Various third-party vendors supporting P&T/DUR functions such as clinical reviews, preferred drug placement and prior authorization
- Whether the state uses separate or combined P&T and DUR committees
- Whether the state announces a treatment will be reviewed
- Whether the state allows meetings with P&T/DUR members prior to the public meeting
- Rules for meetings with members
- Whether they use a PBM's recommendations
- Whether a manufacturer medical science liaison (MSL) presentation has an impact
- Whether draft clinical criteria or committee recommendations are posted before a meeting
- P&T and DUR annual review cycle, meeting format, speaker policies, time allowed to speak, list of members, bylaws, meeting address, links to agendas, and agenda posting timeframes
- Whether the state has a Rare Disease Advisory Council (RDAC)

State-by-state review

COLORADO DRUG UTILIZATION REVIEW BOARD

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Nov 14, 2023	Nov 12, 2024	Aug 8, 2023	Not Yet Reviewed
Date Policy Published	Dec 20, 2023	Dec 1, 2024	Dec 1, 2023	Not Yet Reviewed

- Holds virtual meetings
- Agenda released ~30 days before meeting
- Proposed drug criteria not published in advance of meeting
- Public comment: Allows advance written comments and 24-hour advance registration to offer verbal comments limited to 3 mins/speaker
- Policy completed through DUR, meets quarterly
- FDA approval to Medicaid review ranges from 2-9 months

GEORGIA DRUG UTILIZATION REVIEW BOARD

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Unknown	Unknown	Jan 17, 2024	Not yet reviewed
Date Policy Published	Jan 1, 2024 Revised	Feb 1, 2025 Revised	No criteria published	Not yet reviewed

- In-person DUR review; no virtual option
- Agenda released ~60 days before meeting
- Allows clinical/scientific exchange with manufacturer in advance of DUR
- Meeting packet posted 1 day in advance of DUR
- Public comment: Allows written comments sent via postal mail 5 days in advance and/or register day-of to give 3-minute verbal comments
- Policy completed through DUR, meets quarterly/ as needed
- FDA approval to Medicaid review is around 7 months (Elevidys only)

ILLINOIS DRUG AND THERAPEUTICS COMMITTEE

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Apr 11, 2024	Jan 1, 2025	Oct 5, 2023	Not Yet Reviewed
Date Policy Published	No criteria published	No criteria published	No criteria published	Not Yet Reviewed

- Virtual meeting
- Agenda released ~30 days before meeting
- Manufacturer may submit in advance a 4-page clinical summary
- Public comment: Must register 14 days prior to meeting with 3 minutes for verbal remarks
- Policy completed through D&T, meets quarterly
- FDA approval to Medicaid review ranges from 4-13 months

KANSAS DRUG UTILIZATION REVIEW BOARD

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Aug 14, 2024	Oct 16, 2024	Jul 9, 2023, revised Aug 14, 2024	Not yet reviewed
Date Policy Published	Unknown	Unknown	Unknown	Not yet reviewed

- Virtual and in-person meeting
- Agenda released ~7 days before meeting
- Meeting materials sometimes published 1 day in advance
- Public comment: 7-day advance registration required; no time limit specified
- Policy completed through DUR, meets quarterly
- FDA approval to Medicaid review ranges from 1-18 months

MASSACHUSETTS DRUG UTILIZATION REVIEW BOARD

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Unknown	Unknown	Unknown	Unknown
Date Policy Published	July 31, 2023	Jan 6, 2025	Revision Apr 4, 2025	Revision Apr. 4, 2025

- In-person and virtual
- Agenda released ~7 days before meeting
- Agenda does not list drugs to be reviewed
- Does not publish meeting materials in advance
- Public comment: Restricts to 3 total participants (or a total of 15 minutes) for all agenda topics; no guidelines published — must request and then receive instructions
- Policy completed through DUR, meets quarterly
- Acts as both DUR and P&T

MINNESOTA DRUG FORMULARY COMMITTEE

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Not yet reviewed	Dec 18, 2024	Apr 17, 2024	Dec 7, 2023
Date Policy Published	Not yet reviewed	Revision Mar 2025	Revision Oct 2024	Revision Mar 2025

- Virtual and in-person meeting
- Agenda released ~30 days before meeting
- DFC membership must include at least one rare disease specialist
- Meeting materials with proposed coverage policies published approximately 30 days in advance
- Public comment: Opens once agenda is posted and available up to 15 days post-meeting; in-person verbal testimony limited to 3 minutes/speaker
- Policy published approximately 2 weeks post-meeting
- Policy completed through P&T, meets quarterly
- FDA approval to Medicaid review ranges from pre-approval (Qalsody) to 10 months post-approval

MISSISSIPPI PHARMACY & THERAPEUTICS COMMITTEE

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Not yet reviewed	Oct 22, 2024	Feb 13, 2024	Not yet reviewed
Date Policy Published	Not yet reviewed	No criteria published	Feb 1, 2024	Not reviewed

» In-person meeting with listen-only virtual option

» Agenda released ~30 days before meeting

» Meeting materials and proposed coverage policy published 7 days in advance

» In-person attendance limited to 44 total, with only 1 person per organization

» Public comment: In-person only; register at least 1 day prior; 3 minutes/speaker; limited to manufacturers and patient advocacy organizations

» Policy completed through P&T, meets quarterly

» FDA approval to Medicaid review ranges from 2-8 months

OREGON PHARMACY & THERAPEUTICS COMMITTEE

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Jun 1, 2023	Oct 3, 2024	Feb 1, 2024 and Oct 1, 2024	Aug 1, 2024
Date Policy Published	July 1, 2023	Jan 1, 2025	Dec 1, 2024	Nov 1, 2024

» Virtual meeting

» Agenda released ~60 days before meeting

» Allows an expert physician to participate as an ad hoc P&T member

» Meeting materials published in advance with public comment period prior to P&T meeting

» Public comment: Must register in advance; 3 minutes/speaker, up to 10 minutes per agenda topic; written comments accepted 2 weeks in advance

» Policy completed through P&T, acts as P&T and DUR meets quarterly

» FDA approval to Medicaid review ranges from 2-8 months

VIRGINIA DRUG UTILIZATION REVIEW BOARD

	Skyclarys	Livdelzi	Elevidys	Qalsody
Date Approved	Feb 2023	Aug 2024	Jun 2023 and Jun 2024	Apr 2024
Date Reviewed	Sep 14, 2023	Jan 16, 2025	Jun 13, 2024 and Jan 15, 2025	Not yet reviewed
Date Policy Published	Unknown	No criteria published	Jan 1, 2025	Not yet reviewed

» In-person meeting only

» Agenda released ~30 days before meeting

» Meeting materials and proposed criteria not published in advance

» ELEVIDYS DUR review conducted 1/15/2025 with only 2 days' notice; policy reviewed and voted on for multiple CGTs at this meeting

» Public comment: Register 30 days before meeting; 3 minutes/speaker for P&T, 5 minutes/speaker for DUR

» Policy completed through DUR, meets quarterly

» FDA approval to Medicaid review ranges from 5-12 months

Summary of Findings

All stakeholders — state Medicaid programs and their contract partners, patients and their families, caregivers, patient advocates, treating physicians and drug manufacturers, among others — have an interest in improving the journey and health outcomes for patients with rare diseases. LMDDAN's research of a diverse geographic sample of state Medicaid review processes for four FDA-approved rare disease drugs shines a light on how the processes vary from state to state.

Disparities include how patients, patient advocates and treating physicians can share their expertise to best inform a coverage policy; the level of engagement with the innovator/manufacturer in a clinical and scientific discussion; and, the timing and publication of information.

Specific findings include:

» **State review of the select four drugs at a P&T/DUR Committee:** States did not consistently review all four drugs. Notably, Qalsody was reviewed by only two states (Minnesota and Oregon) and Elevidys was reviewed by eight of nine states through a P&T/DUR Committee process. One state, Massachusetts, did not review any of the four rare disease drugs through a P&T/DUR committee process.

» **Time from FDA approval to a drug's review by a P&T/DUR Committee:** States ranged in their review of drugs from one month to 18 months post-FDA approval, with the most common P&T/DUR Committee held eight to 12 months post-drug approval.

» **Advance public posting of P&T/DUR Committee meeting materials:**

– Agenda: Generally, half of states publish detailed agendas approximately 30 days in advance of a meeting. Some states post an agenda seven days or fewer prior to a meeting. One state, Virginia, posted a review of gene therapies, including Elevidys, with two days' notice.

  - Meeting materials and proposed drug coverage policy: Five states published materials in advance of the meeting ranging from one day prior to 60 days in advance. Three states — Colorado, Illinois and Virginia — reviewed rare disease drugs without publishing meeting materials or draft coverage policies in advance of the meeting.

» **Meeting accessibility:** Six states offer a virtual option for stakeholders to participate. Three states only hold the meeting in-person, limiting public engagement.

» **Stakeholder expertise:** All states offer a public comment process, allowing written testimony or live remarks, generally limited to three minutes per speaker. One state, Massachusetts, limits the public comment portion of the meeting to a maximum of 15 minutes. Another state, Virginia, held at least one public comment session after the DUR voted on the drug coverage policies. Notably, two states offer unique opportunities for a substantive clinical and scientific exchange. For example, Minnesota and Oregon allow a subject matter expert or rare disease expert to serve as a member of the P&T Committee during a drug review. Additionally, Georgia offers the drug innovator an opportunity to participate in a clinical review of the drug weeks prior to the P&T meeting. Both processes, while different, ultimately serve to ensure a drug coverage policy is informed by the science, clinical data and etiology and progression of disease.

» **Time to drug coverage policy posted after P&T/DUR Committee review:** It took up to 12 months for specific drug coverage polices to be posted after a P&T and DUR meeting, with most states posting policies three to six months after.

Recommendations

This research demonstrates that there is significant variation among state Medicaid programs. Common guidelines or best practices should be adopted to ensure a more consistent drug review process from state to state. Recommended actions include:

» **Require rare disease expertise to inform drug coverage reviews and policy development**

  - Invite a rare disease physician expert to offer their clinical expertise to inform disease background and treatment approach when developing a coverage policy for a rare disease drug
  - Offer an opportunity for the drug innovator to present clinical and scientific data

» **Improve transparency and stakeholder input in Medicaid P&T and DUR meetings**

  - Publish a step-by-step explanation of the DUR/P&T Committee process
  - Ensure DUR/P&T Committee members do not have conflict of interests
  - Provide at least 30 days' advance notice of meeting date, specific agenda topics (i.e. drugs to be reviewed), and draft coverage policies for discussion
  - Offer virtual interactive access to DUR/P&T committee meetings, or similar body, to encourage and allow diverse stakeholder participation, including clinician, patient, caregiver, patient advocate and manufacturer
  - Allow public testimony during each individual drug review, and prior to DUR/P&T vote on coverage policy
  - Allow adequate time for individual public comment (i.e. five minutes), and do not limit the number of stakeholders
  - Publish meeting minutes and drug coverage policies within 14 days of meeting

» **Align drug coverage policies to the FDA-approved, medically accepted indication**

- Publish coverage policies to a central repository on the state's Medicaid webpage
- Share coverage policies with providers and with the state's contracted managed care organizations (MCOs) to ensure consistent patient access within the state

Collectively, adoption of these best practices will ensure a more transparent and accessible platform for all stakeholders to inform the development of drug specific coverage policies in the best interest of rare disease patients.