

NEW JERSEY DRUG UTILIZATION REVIEW BOARD  
VIRTUAL PLATFORM

October 18, 2023

<http://www.state.nj.us/humanservices/dmahs/boards/durb/>

AGENDA

- I. Call to order in accordance with New Jersey Open Public Meeting Act
- II. Roll Call
- III. Review of meeting transcript for July 19, 2023, meeting
- IV. Review of draft meeting summary for July 19, 2023, meeting (pages 3-7)
- V. Secretary's report (page 8)
- VI. Old Business
  - A. Proposed addendum to Biologic Receptor Modifiers (BRMs) for plaque psoriasis protocol (pages 9-11)
  - B. Risk Evaluation and Mitigation Strategy (REMS) programs in institutions (pages 12-16)
- VII. New Business
  - A. Proposed protocol for Kanuma (sebelipase alfa) [page 17]
  - B. Proposed protocol for Vyjuvek (beremagene geperpavec) [pages 18-19]
  - C. Proposed addendum to Duchenne Muscular Dystrophy products protocol (pages 20-22)
- VIII. A. Informational Highlights/Reports
  1. Gainwell Technologies/NJ MCO 2<sup>nd</sup> Quarter 2023 Prior Authorization Report (page 23)
  2. Summary of DURB Action Items (pages 24-25)
  3. (a) DHS, DHSS and MCO Programs Top Drugs Report/Physicians Administered Drugs (by amount paid and by category)  
(b) Antiviral drugs by amount paid
- B. Medication information:
  1. Opioid National Drug Code and Oral MME Conversion File Update  
<https://www.cdc.gov/opioids/data-resources/index.html>
  2. Long COVID Symptoms May Emerge Months After Infection  
[https://www.medpagetoday.com/neurology/longcovid/105849?xid=nl\\_mpt\\_DHE\\_2023-08-10&eun=g2076570d0r&utm\\_source=Sailthru&utm\\_medium=email&utm\\_campaign=Daily%20Headlines%20Evening%202023-08-10&utm\\_term=NL\\_Daily\\_DHE\\_dual-gmail-definition](https://www.medpagetoday.com/neurology/longcovid/105849?xid=nl_mpt_DHE_2023-08-10&eun=g2076570d0r&utm_source=Sailthru&utm_medium=email&utm_campaign=Daily%20Headlines%20Evening%202023-08-10&utm_term=NL_Daily_DHE_dual-gmail-definition)
  3. Dementia Risk Linked With Cumulative Heartburn Med Use, Analysis Suggests  
[https://www.medpagetoday.com/neurology/dementia/105827?xid=nl\\_mpt\\_DHE\\_2023-08-09&eun=g2076570d0r&utm\\_source=Sailthru&utm\\_medium=email&utm\\_campaign=Daily%20Headlines%20Evening%202023-08-09&utm\\_term=NL\\_Daily\\_DHE\\_dual-gmail-definition](https://www.medpagetoday.com/neurology/dementia/105827?xid=nl_mpt_DHE_2023-08-09&eun=g2076570d0r&utm_source=Sailthru&utm_medium=email&utm_campaign=Daily%20Headlines%20Evening%202023-08-09&utm_term=NL_Daily_DHE_dual-gmail-definition)

4. Poorer Neighborhoods Linked to Higher Asthma Rates in Kids  
[https://www.drugs.com/news/poorer-neighborhoods-linked-higher-asthma-rates-kids-114721.html?utm\\_source=ddc&utm\\_medium=email&utm\\_campaign=Daily+Mednews+++September+1++2023&utm\\_content=Poorer+Neighborhoods+Linked+to+Higher+Asthma+Rates+in+Kids&has\\_h2=2f2d27208920fb433cf3207028cab550](https://www.drugs.com/news/poorer-neighborhoods-linked-higher-asthma-rates-kids-114721.html?utm_source=ddc&utm_medium=email&utm_campaign=Daily+Mednews+++September+1++2023&utm_content=Poorer+Neighborhoods+Linked+to+Higher+Asthma+Rates+in+Kids&has_h2=2f2d27208920fb433cf3207028cab550)
5. Certain SSRIs May Increase Arrhythmia Risk in Select Patients  
<https://www.medpagetoday.com/psychiatry/depression/106115#:~:text=Therapeutic%20doses%20of%20some%20selective,to%20a%20Norwegian%20cohort%20study>

## July 19, 2023 DURB Meeting Summary (draft)

Issue	Action	Notes
Roll Call		<p><u>Present:</u> Dr. Swee, Dr. Gochfeld, Dr. Moynihan, Dr. Barberio, Ms. Olson, Dr. Lind (ex-officio)</p> <p><u>Unable to attend:</u> Dr. Marcus, Mr. Schafer</p>
Dr. Swee's pre meeting announcement		<p>Dr. Swee called the meeting to order by reading the following statement as required for the Board's meetings:</p> <p>In compliance with Chapter 231 of the public laws of 1975, notice of this meeting was given by way of filings in the Trenton Times, the Star Ledger and Atlantic City Press.</p>
Review of Minutes	Approved	<p>Minutes from April 19, 2023, meeting was reviewed and approved. The approved meeting summary will also be posted on the DURB website at:</p> <p><a href="http://nj.gov/humanservices/dmahs/boards/durb/meeting/index.html">http://nj.gov/humanservices/dmahs/boards/durb/meeting/index.html</a></p>
Secretary's Report		<ul style="list-style-type: none"> <li>- The Commissioners have signed off on DURB-recommended protocol for April 2022 and October 2022.</li> <li>- The Department is working with the Commissioners to also sign off on DURB recommended protocols for , January 2022, and April 2023.</li> <li>- The DHS Commissioner's office is in contact with NJ PHARMA (Pharmaceutical Association) regarding potential replacement of a board member. We are awaiting information on the future appointee.</li> <li>- Dr. Lind informed the Board that the Department of Health is working on a replacement for their representative who was lost six years ago.</li> <li>- Dr. Swee pointed out that not all Board members have been contacted by professional associations in support of reappointment.</li> <li>- The Board raised no concerns about the medical necessity forms for Skysona and Zynteglo which they reviewed.</li> </ul>

## July 19, 2023 DURB Meeting Summary (draft)

Issue	Action	Notes
<b>Old Business</b>		
(A)MCO response to PA denials report	Continue to monitor	Dr. Swee commented on the variances between the response times among the Managed Care Organizations (MCOs).
(B) MCOs churn rate request report	Continue to monitor	The Board reviewed the churn rate report for the MCOs. Dr. Swee observed that it has dropped substantially which is good for the patients and healthcare. The Board requested that this should be an annual report.
(C) Legembi and Aduhelm utilization report		There was no utilization for these products from 2022 thru June 2023. The Board recommended to keep the review on its future meeting agenda.
(D)Report for non-formulary denials for antidiabetic medications	Continue to monitor	The Board reviewed a prior authorization denials report (by therapeutic class) of antidiabetics. Dr. Swee commented on the differences in non-formulary status of some of the medications. He announced that the Board will review the formulary of the MCOs to determine why one in six or one in seven patients are denied medications due to non-formulary issues.
(E ) Hemgenix use in pediatric patients		The Board reviewed a letter from CSL Behring, the manufacturer of Hemgenix, explaining that they had not done any studies on pediatric patients. Dr. Swee announced that the Board is in the process or reaching out to pediatric and family physician groups to see what they are doing about that and will update the public when that information is available.
(F) Calcitonin gene-related peptide (CGRP) inhibitors utilization	Continue to monitor	The Board reviewed a utilization report for CGRP inhibitors. The report showed overall utilization of 29% and 2% for FFS and MCOs respectively. The Board requested another report in six months.
(G) Summary of DURB suggested changes to proposed Livmarli protocol		The Board reviewed their suggested changes for proposed Livmarli protocol. They had no further comments.

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## July 19, 2023 DURB Meeting Summary (draft)

Issue	Action	Notes
<b>New Business</b>		
(A) Proposed protocol for CAR T-cell products	Approved	<p>The Board reviewed a proposed protocol for chimeric antigen receptor (CAR T-cell) products. These products are used as targeted, personalized therapy that contains patients' analogous T cells reengineered to fight cancer. Dr. Swee had questions about the process of delivering the medical necessity forms and contacting the prescribing physicians at institutions, especially with the temporary nature of residents and fellows. Dr. Emenike responded that the same process as in the outpatient will apply. He doubted that this class of medications will be prescribed by residents. Ms. Olson wondered who should be enrolled in the Risk Evaluation and Mitigation Strategy (REMS) program, the hospital or prescriber. Mr. Vaccaro informed the Board that he will provide information at the next meeting that will give the Board an understanding of REMS-related billing and other processes involved.</p> <p>The Board recommended the protocol</p>
(B) Proposed protocol for Qalsody	Approved	<p>The Board reviewed a proposed protocol for Qalsody (tofersen), a product indicated for the treatment of Amyotrophic Lateral Sclerosis (ALS) in adults. Dr. Swee requested Dr. Moynihan's opinion on Medicaid's reimbursement for the product. She responded that they pay for similar products used for ALS.</p> <p>The Board recommended the protocol.</p>
(C) Proposed addendum for biologic respond modifiers (BRMs) protocol	Tabled	<p>The Board reviewed a proposed addendum for BRMs used in the treatment of plaque psoriasis protocol. Dr. Moynihan enquired about the process if a patient also had psoriatic arthritis, or other overlapping diseases. Dr. Emenike responded that the State will honor the claim if there are no other clinical issues. Dr. Swee wondered what protocol would be applied. Dr. Emenike explained that the protocol was intended for plaque psoriasis due to the constant exposure to direct-to-consumer advertising but did not foresee a problem with occasional overlaps. Dr. Gochfeld was concerned that since this is not a life-threatening illness, the requirement for one conventional drug trial prior to use of the BRMs was too low a hurdle. Dr. Swee also felt that only 3 months trial of topical corticosteroids was a low threshold too. He and Dr. Moynihan suggested seeking guidance from a dermatologist. Dr. Lind suggested that the leniency in the protocol may not be an issue since the MCO's</p>

## July 19, 2023 DURB Meeting Summary (draft)

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		<p>formularies could limit use. The Board however decided to proceed with the dermatology consult. The protocol was tabled for the next meeting.</p>																												
<p><b>Informational Highlights/Reports</b></p>																														
<p>1. Fee-for-Service/MCO Prior Authorization Report</p> <p>2. Summary of DURB Actions/Recommendations</p>	<p>Continue to monitor.</p>	<p>The percentage of prior authorization requests relative to total claims and denials associated with the PAs for the 1<sup>st</sup> quarter 2023 are shown below.</p> <table border="1" data-bbox="848 526 1814 795"> <thead> <tr> <th>Plan</th> <th>(%) PA Requests of claims</th> <th>Denial (%)</th> <th>% w/o NF*</th> </tr> </thead> <tbody> <tr> <td>FFS</td> <td>0.6</td> <td>7</td> <td>7</td> </tr> <tr> <td>Aetna</td> <td>1</td> <td>37</td> <td>13.2</td> </tr> <tr> <td>Amerigroup</td> <td>0.9</td> <td>36</td> <td>16</td> </tr> <tr> <td>Horizon</td> <td>0.8</td> <td>36</td> <td>12</td> </tr> <tr> <td>UHC</td> <td>1</td> <td>45</td> <td>16</td> </tr> <tr> <td>WellCare</td> <td>0.8</td> <td>33</td> <td>10</td> </tr> </tbody> </table> <p>NF = Non formulary Dr. Swee again expressed concern over United Healthcare's (UHC) high denial rate. There was no further comments from board members.</p> <p>The Board reviewed a summary of their actions from previous meetings (July 2022 thru April 2023). There were no comments.</p>	Plan	(%) PA Requests of claims	Denial (%)	% w/o NF*	FFS	0.6	7	7	Aetna	1	37	13.2	Amerigroup	0.9	36	16	Horizon	0.8	36	12	UHC	1	45	16	WellCare	0.8	33	10
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<p>3. DHS/DHSS/MCO Programs Top Drugs Report</p>		<p>Top drugs report for May 2023 (FFS) and April 2023 (MCOs) was provided for review. Drug expenditures during the reporting period is noted below:</p> <table border="1" data-bbox="848 1256 1814 1429"> <thead> <tr> <th>Plan</th> <th>Month Reported</th> <th>Top Drugs</th> <th>Total</th> </tr> </thead> <tbody> <tr> <td>FFS</td> <td>May 2023</td> <td>\$11,158,169</td> <td>\$11,509,968</td> </tr> <tr> <td>MCOs</td> <td>April 2023</td> <td>\$115,944,192</td> <td>\$160,723,222</td> </tr> </tbody> </table>	Plan	Month Reported	Top Drugs	Total	FFS	May 2023	\$11,158,169	\$11,509,968	MCOs	April 2023	\$115,944,192	\$160,723,222																
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## July 19, 2023 DURB Meeting Summary (draft)

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4. Medication Information		<p>Medical information was provided with links for further reading on the topics below:</p> <ol style="list-style-type: none"> <li>1. Weighing the Consequences of Weight-Loss Drugs</li> <li>2. FDA Approves First Oral Antiviral for Treatment of COVID-19 in Adults</li> <li>3. House Passes Bill to Address Fentanyl Overdoses</li> <li>4. COVID-19 Vaccines information</li> </ol>
Follow-up items:		<ol style="list-style-type: none"> <li>A. Present the MCOs churn rate report annually</li> <li>B. Present the CGRP utilization report in 6 months</li> <li>C. Billing and REMS process in inpatient environment (Ed Vaccaro.)</li> <li>D. MCO formulary comparison report</li> </ol>

## NEW JERSEY DRUG UTILIZATION REVIEW BOARD

October 18, 2023

### Secretary's Report:

1. The department is working with the Commissioners to review and sign off on DURB-recommended protocols for:
  - January 2023
  - April 2023
  - July 2023
2. The DHS Commissioner is reviewing the recommended changes for the reappointment and replacement of DURB members
3. Proposed dates for 2024 DURB meetings:
  - Wednesday, January 24, 2024
  - Wednesday, April 17, 2024
  - Wednesday, July 17, 2024
  - Wednesday, October 16, 2024



# Addendum to the Protocol for Biological Response Modifiers in the Treatment of Plaque Psoriasis

October 2023

Approved July 2021

## Addendum:

- Addition of new product, Sotyktu<sup>®</sup> (approved September 2022)
- Add mild Plaque Psoriasis for Otezla (approved December 2021)
- Add Enbrel for  $\geq 4$  years old (per PI)
- Add Humira for  $\geq 4$  years old (Per 2020 American Academy of Dermatology psoriasis in pediatric patients guidelines)
- Add FDA approved biosimilars of above products

Amjevita (adalimumab-atto)

Avsola (infliximab)

Cimzia (certolizumab)

Cosentyx (secukinumab) [ $\geq 6$  years old]

Enbrel (etanercept) [ $\geq 4$  years old]

Humira (adalimumab) [ $\geq 4$  years old]

Ilumya (tildrakizumab)

Inflectra (infliximab)

Otezla (apremilast)

Remicade (infliximab)

Renflexis (infliximab)

Siliq (bradalumab)

Skyrizi (risankizimab-rzaa)

Sotyktu (deucravacitinib)

Stelara (ustekinumab) [ $\geq 6$  years old]

Taltz (ixekizumab) [ $\geq 6$  years old]

Tremfya (guselkumab)

## Background:

*Biologic response modifiers (BRMs), also known as immunomodulators, are the class of medications that target the disease-causing mechanism. They are used in autoimmune diseases as first-line medications or after the failure of conventional agents. Serious infections are the most severe complications and require screening before initiation, and monitoring while patients are taking the medications.*

## Criteria for Approval:

A. Patient meets ALL the following:

1. For all drugs except Otezla: Diagnosis of moderate to severe plaque psoriasis
2. For Otezla: Diagnosis of plaque psoriasis

3. Medication is used for an adult patient except where otherwise indicated
4. Patient must have disease affecting crucial body areas such as hands, feet, face, or genitals  
OR either a or b:
  - a. For all drug requests except Otezla: Patient must have clinical documentation of a diagnosis of moderate to severe plaque psoriasis characterized by greater than or equal to 3% body surface involved
  - b. For Otezla requests: Patient must have clinical documentation of a diagnosis of mild to severe plaque psoriasis characterized by greater than or equal to 2% body surface involved
5. History of trial and failure of at least **TWO** of the following conventional therapies at maximally tolerated doses (for **TWO** months) each unless contraindicated or clinically significant adverse effects are experienced (document drug, date, and duration of trial):
  - i. Acitretin
  - ii. Topical Vitamin D analogs (e.g., calcitriol)
  - iii. Topical corticosteroids
  - iv. Calcineurin inhibitors (e.g., tacrolimus or pimecrolimus)
  - v. Topical retinoic acid derivatives
  - vi. Phototherapy
6. Initial prescription is written by or in consultation with a dermatologist
7. Patient does not have any contraindications to therapy
8. Patient is not receiving medication with another BRM
9. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence
10. Prior to initiation of therapy, patient is tested for tuberculosis (TB) [where applicable]
11. Weight must be received for drugs that have weight-based dosing for any dose change request

**Continuation of therapy:**

1. Documentation of positive clinical response to therapy
2. Patient is not receiving medication with another BRM
3. Patient is monitored for active TB during treatment (where applicable)
4. Patient is monitored for lymphoma and other malignancies during treatment (where applicable)

**Note:**

**Suicidal ideation and behavior, including completed suicides, have occurred in patients treated with Siliq.**

**References:**

1. Cimzia [packet insert] UCB, Inc. 1950 Lake Park Drive Smyrna, GA 30080. September 2019
2. Cosentyx [packet insert] Novartis Pharmaceuticals Corporation East Hanover, NJ 07936. June 2020
3. Enbrel [packet insert] Amgen. Thousand Oaks, CA 91320. April 2021
4. Humira [packet insert] AbbVie Inc. North Chicago, IL 60064. February 2121
5. Ilumya [packet insert] Merck & Co. Inc., White House Station, NJ 08889. March 2018
6. Otezla [packet insert] Amgen Inc. Thousand Oaks, CA 91320. June 2020
7. Remicade [packet insert] Janssen Biotech, Inc. Horsham, PA 19044. November 2013
8. Skyrizi [packet insert] AbbVie Inc. North Chicago, IL 60064. April 2021
9. Siliq [packet insert] Bausch Health US, LLC Bridgewater, NJ 08807. April 2020
10. Sotyktu [packet insert] Bristol-Myers Squibb Company. Princeton, NJ 08543. September 2022
11. Stelara [packet insert] Janssen Biotech, Inc., Horsham, PA 19044. December 2020
12. Taltz [packet insert] Eli Lilly and Company, Indianapolis, IN 46285. March 2021
13. Tremfya [packet insert] Janssen Biotech, Inc., Horsham, PA 19044. July 2020
14. Menter A et al. Joint AAD-NPF guidelines of care for the management and treatment of psoriasis with biologics. *A Am Acad Dermatol* 2019;80:1029-72
15. Pardasani AG, Feldman SR, Clark AR. Treatment of psoriasis: An algorithm-based approach for primary care physicians. *Am Fam Physician* 2000;61(3):725-733.
16. Clinical Pharmacology (online database). Tampa FL: Gold Standard Inc.: 2019. Updated periodically
17. Feldman SR. Treatment of psoriasis in adults. UpToDate February 2021. Accessed online 5.3.21 @ <https://www.uptodate.com/contents/treatment-of-psoriasis-in-adults>
18. Kim WB. Diagnosis and management of psoriasis. *Can Fam Physician*. 2017 Apr; 63(4): 278–285
19. Wu, JJ. Contemporary Management of Moderate to Severe Psoriasis. *Am J Manag Care*. 2017;23:S403-S416
20. Menter A, Cordoro KM, Davis DMR, et al. Joint American Academy of Dermatology – National Psoriasis Foundation guidelines of care for the management and treatment of psoriasis in pediatric patients. *J Am Acad Dermatol*. January 2020;82:161-201.

# RISK EVALUATION AND MITIGATION STRATEGY (REMS)

A REMS is a program established under the Food and Drug Administration Amendments Act of 2007 (FDAAA). The FDAAA grants the FDA the authority to require a drug manufacturer to develop and implement a REMS if the FDA determines a REMS is necessary to ensure that the benefit of a drug outweighs the risks. The FDA considers risk management to be the continuing process of minimizing risks throughout a product's life cycle to optimize its benefit-risk balance. For more information, please go to <http://www.fda.gov/Drugs/DrugSafety/PostmarketDrugSafetyInformationforPatientsandProviders/ucm111350.htm>.

## REMS ELEMENTS

A REMS may include one or more of the following: A Medication Guide or patient package insert for patients, a Communication Plan for healthcare providers, and Elements to Assure Safe Use (ETASU), which often involve some form of restricted distribution and/or evidence of safe-use conditions.

### Medication Guide

A Medication Guide provides FDA-approved patient labeling. A Medication Guide can be required if the FDA determines one or more of the following apply:

- Patient labeling could help prevent serious adverse events.
- The product has serious risks that could affect a patient's decision to use or continue to use the drug.
- Patient adherence to directions is crucial to product effectiveness.

A Medication Guide must be used as a review tool in counseling the patient on the risks of the prescribed medicine. Healthcare providers are required to dispense a Medication Guide with each prescription.

### Communication Plan

A Communication Plan is targeted at healthcare providers to support implementation of the REMS. The communication plan may include sending letters to healthcare providers; disseminating information about REMS elements to encourage implementation by healthcare providers or to explain certain safety protocols, such as medical monitoring by periodic laboratory tests; or disseminating information to healthcare providers through professional societies about any serious risks of the drug and any protocol to assure safe use.

### Elements to Assure Safe Use (ETASU)

ETASU are intended to provide safe access for patients to drugs with known serious risks that would otherwise be unavailable. Required ETASU are put in place to mitigate a specific serious risk listed in the labeling of a drug. ETASU can include one or more of the following requirements:

- Prescribers have particular training or experience, or are specially certified
- Certification of dispensers
- Drug administration restricted to certain healthcare settings
- Documentation of safe-use conditions prior to dispensing
- Monitoring of patients
- Enrollment of patients in a registry

**Failing third-party REMS audit.** Vigabatrin, an antiseizure medication used to treat infantile spasms and refractory complex partial seizures, carries a Boxed Warning for the risk of permanent vision loss. For inpatient pharmacies to dispense vigabatrin, they must be enrolled in the REMS program, and staff must be trained in the program requirements. Staff must verify that the prescriber is certified in the program and that the patient is enrolled in the program. Staff must obtain authorization to dispense the drug by contacting the program by phone or [logging in online](#). Staff must also document the prescriber identification (ID) number, patient ID number, and the authorization code that are all assigned by the Vigabatrin REMS Program, in a paper/digital logbook or in the electronic health record (EHR) within 15 days of the patient's admission.

**1. Do I, or does my healthcare setting or pharmacy, have to enroll in a certain program?**

Some REMS require that a pharmacy or healthcare setting be certified to be able to receive or dispense the medication. To fulfill this requirement, the pharmacy or health care setting may identify an authorized representative to enroll on behalf of the pharmacy or setting. However, in some cases, every pharmacist who dispenses the medication may be required to complete training to meet this requirement. Individual pharmacists or pharmacy staff may also be required to enroll to obtain authorization to dispense. Not every program will require these steps, so it is important to check the [REMS@FDA website](#) or the manufacturer's REMS website for a complete list of requirements for the specific medication to be dispensed.

**1. Does the patient have to be enrolled in a certain program?**

Some REMS require the patient to be enrolled. In most cases, health care providers enroll patients. The pharmacist may need to verify that the patient is enrolled before dispensing the medication. Depending on the program, this may require a phone call to the REMS call center,

verification through the specific product REMS website, or verification that is built into the pharmacy management system.

2. **Does the health care provider have to be enrolled in a certain program?**

Some REMS will require the health care provider to be certified or enrolled. The pharmacist may need to verify that the health care provider is certified or enrolled before dispensing the medication.

Depending on the program, this may require a phone call to the REMS call center, verification through the specific product REMS website, or verification that is built into the pharmacy management system.

3. **Do I have to dispense a Medication Guide or any other material to the patient?**

All [Medication Guides](#) are approved as part of labeling, but only a small number of Medication Guides are included as part of REMS.

REMS may also provide additional patient-focused materials for distribution or may require patient counseling by pharmacists to ensure that patients are educated about specific risks of the medication.

Pharmacists may need to answer patient questions that arise after the patient reads a Medication Guide, other patient focused materials, and/or during patient counseling.

4. **Is there monitoring involved with medications that are under a REMS?**

Some REMS medications do require documentation of specific lab test results or some other form of patient monitoring. In the case of a drug that requires specific lab test results or some other type of monitoring prior to dispensing, the pharmacist is responsible for verifying that the lab test or other monitoring tests have been completed before filling or refilling a medication. Depending on the program, verification of the specific requirement may require a phone call to the REMS call center, verification through the specific product REMS website, or verification that is built into the pharmacy management system. If the information cannot be verified, the pharmacist may need to contact a health care provider's office to ensure the lab test or other monitoring requirement was ordered or conducted.

5. **Can I order a medication with a REMS through my usual supplier?**

Check with the individual REMS to confirm the distribution requirements, as some REMS medications can only be obtained from a specific supplier or may not be able to be delivered to all pharmacy settings.

**6. I understand pharmacists may have to verify safe use conditions. How is that done?**

Pharmacists play a key role in REMS as the last checkpoint before patients receive their medication. In many REMS with participant requirements, pharmacists are asked to go to a website or a contact a call center to verify that certain safe use conditions are in place prior to dispensing. For example, pharmacists may need to confirm that the prescriber or patient is enrolled or the patient has undergone laboratory monitoring. Certain programs verify safe use conditions directly through the pharmacy management system using an electronic verification system, sometimes referred to as a “switch.”

**7. Am I required to complete training?**

Some REMS have required training for pharmacists which may include passing a knowledge assessment test. For most REMS, the authorized pharmacy representative is required to take the initial training that is part of the certification process for pharmacies and to make sure all pharmacists are trained. Information about training or any other requirements can be found at [REMS@FDA](mailto:REMS@FDA), in product labeling, or on REMS-specific websites.

**8. Are there restrictions on the amount of medication that can be dispensed?**

Some REMS medications have additional restrictions on the number of refills allowed or the days supply dispensed. For example, a medication with a risk of teratogenicity (ability to cause birth defects) may be limited to a one-month supply at a time and may not be refilled unless certain criteria are met, such as verification of a negative pregnancy test.

**9. My hospital /health care setting isn't certified in any REMS, so how does REMS affect me?**

Even if your hospital/health care setting isn't certified to dispense a REMS drug, a patient who is on a REMS medication may be admitted to your hospital or to your emergency department. For example, if you work in an emergency department, you may be treating a patient who experienced a serious adverse event related to a drug he or she is already taking that has REMS requirements. Patients on a REMS drug may also be admitted to your hospital for an unrelated reason and may need to continue treatment on their REMS drug. If you work in an inpatient setting, it may be important for you to understand that your hospital may not stock certain REMS medications. The approved prescribing information is a good resource for medication information as well as information about specific requirements to continue that patient on a REMS medication. Approved prescribing information can be found at [Drugs@FDA](mailto:Drugs@FDA) or [DailyMed](https://www.dailymed.gov). Information about REMS requirements

can be found at [REMS@FDA](mailto:REMS@FDA), in product labeling, or on REMS-specific websites.

**10. Am I, or is my pharmacy, subject to an audit?**

REMS are subject to assessment plans which help FDA determine if the REMS is meeting its goals. If the REMS has pharmacy requirements, the pharmacy may be subject to an audit by FDA, the manufacturer, or a third party on behalf of the manufacturer, to assess if the pharmacy is meeting requirements.

**11. How does FDA address the potential administrative burden of REMS on health care providers and dispensers?**

REMS requirements have raised concerns about the administrative burdens placed on already busy health care providers that might increase the amount of time before patients can start taking needed medicines. FDA makes every effort to make REMS requirements the least burdensome they can possibly be, and FDA has the authority to require the manufacturer to modify the REMS to minimize the burden on the health care delivery system of complying with the REMS. The goal is to maintain patient access while still preserving safe use of a drug.

**12. Where do I go if I have questions?**

If you have questions about a specific REMS you can [contact FDA](#) or contact the REMS program directly.

**13. How can I provide feedback?**

Pharmacist feedback is valued by FDA. If you have feedback or questions about a specific REMS, you can [contact FDA](#). Additionally, you can report drug errors, adverse events, or other safety aspects related to REMS medications to [Medwatch](#).



## Proposed Protocol for Kanuma® (sebelipase alfa)

October 2023

**Background:** *Lysosomal acid lipase deficiency (LAL-D) is a rare, autosomal recessive lysosomal storage disorder associated with functional mutations in the LAL gene (LIPA) that cause a deficiency or absence of LAL activity. It is characterized by intracellular accumulation of cholesteryl esters and triglycerides (TGs) and multisystem involvement.*

*Kanuma is a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme indicated for the treatment of patients with a diagnosis of LAL-D.*

### Criteria for approval:

1. Patient is 1 month or older; AND
2. Patient has a diagnosis of LAL-D; AND
3. Diagnosis of LAL-D is confirmed by:
  - a. Enzyme assay demonstrating deficiency of LAL activity; OR
  - b. Documented molecular genetic test showing mutations in the lysosomal acid type (LIPA) gene
4. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

### Continuation of therapy:

1. Patient is responding positively to therapy as evidenced by documentation of clinical response which may include:
  - a. For members with rapidly progressive disease presenting within first 6 months of life: continued survival
  - b. There is improvement in other parameters related to LAL deficiency, including decrease in low-density lipoprotein cholesterol (LDL-C), non-high-density lipoprotein cholesterol (non-HDL-C), or triglycerides; increase in HDL-C, etc.
2. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

### References:

1. Kanuma [prescribing information]. Alexion Pharmaceuticals Inc. Cheshire, CT 06410 April 2015
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2020. Updated periodically
3. Burton BK, Feillet F, et al: Sebelipase alfa in children and adults with lysosomal acid lipase deficiency: Final results of the ARISE study. *Journal of Hepatology* 2022 vol. 76:577-587
4. Pastores GM, Hughes DA. Lysosomal Acid Lipase Deficiency: Therapeutic Options. *Drug Design, Development and Therapy* 2020;14 591-601

## Proposed Protocol for Vyjuvek® (beremagene geperpavec-svdt)

October 2023

**Background:** *Dystrophic epidermolysis bullosa (DEB) is one of the major types of EB, a rare hereditary group of trauma-induced blistering skin disorders. DEB is caused by inherited pathogenic variants in the COL7A1 gene, which encodes type VII collagen, the major component of anchoring fibrils which maintain adhesion between the outer epidermis and underlying dermis.*

*Vyjuvek is a herpes-simplex virus type 1 (HSV-1) vector-based gene therapy indicated for the treatment of wounds in patients 6 months of age and older with dystrophic epidermolysis bullosa with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene.*

### Criteria for approval:

1. Patient is 6 months or older
2. Patient has a diagnosis of DEB with documentation of mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene. Diagnosis is confirmed by ONE of the following:
  - a. Skin biopsy for immunofluorescence mapping
  - b. Transmission electron microscopy
  - c. Genetic testing
3. Patient has at least one open wound that is not infected
4. Patient does not have current evidence or history of squamous cell carcinoma (SCC) in the area to be treated
5. Medication is prescribed by or in consultation with a dermatologist specializing in the treatment of DEB
6. Medication will be applied by a healthcare professional
7. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

### Continuation of therapy:

1. Patient had a positive clinical response to therapy (e.g., decrease in wound size, increase in granulation tissue, complete wound closure)
2. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

References:

1. Vyjuvek [prescribing information]. Krystal Biotech Inc. Pittsburgh, PA 15203 May 2023
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2020. Updated periodically
3. Hou P et al. Innovations in the Treatment of Dystrophic Epidermolysis Bullosa (DEB): Current Landscape and Prospects. Therapeutics and Clinical Risk Management 2023:19
4. Has C, Liu L, Bolling MC, et al. Clinical practice guidelines for laboratory diagnosis of epidermolysis bullosa. Br J Dermatol 2020; 182:574

# Proposed Addendum to Protocol for Duchenne Muscular Dystrophy Products

October 2023

Approved July 2020

Updated July 2021 - Added viltolarsen (Viltepso®) – FDA-approved in August 2020

Updated October 2021:

- a. Added casimersen (Amondys 45) – FDA-approved in February 2021
- b. Changed name of protocol to “Protocol for Duchenne Muscular Dystrophy Products”

Exondys 51® (eteplirsen)

Vyondys 53® (golodirsen)

Viltepso® (viltolarsen)

Amondys 45® (casimersen)

## Addendum:

Addition of Elevidys® (delandistrogene moexeparvovec-rokl) – FDA-approved July 22, 2023

## Background:

Eteplirsen (Exondys 51)® is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Golodirsen (Vyondys 53®) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

Viltolarsen (Viltepso®) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

Casimersen (Amondys 45®) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 skipping.

Delandistrogene moxeparvovec-rokl (Elevidys®) is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene.

**Limitations: This indication is approved under accelerated approval based on expression of Elevidys microdystrophin in skeletal muscle observed in patients treated with Elevidys. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s.)**

## Criteria for Approval:

1. Patient must have the diagnosis of Duchenne Muscular Dystrophy (DMD).
2. Submission of medical records including the following:
  - a. For Exondys 51: Genetic testing confirming the patient has a mutation of the DMD gene that is amenable to exon 51 skipping.
  - b. For Vyondys 53 and Viltepso: Genetic testing confirming the patient has a mutation of the DMD gene that is amenable to exon 53 skipping.
  - c. For Amondys 45: Genetic testing confirming the patient has a mutation of the DMD gene that is amenable to exon 45 skipping.
  - d. For Elevidys:
    - i. Genetic testing with either a confirmed frameshift mutation, or a premature stop codon mutation between exons 18 to 58 in the DMD gene.
    - ii. Patient does not have any deletion in exon 8 and/or exon 9 in the DMD gene
  - e. Baseline renal function tests (i.e., glomerular filtration rate GFR) as required by medication's label
3. Patient has been stable on a systemic corticosteroid regimen for at least 12 weeks, unless contraindicated or experienced significant adverse effects (must receive documentation)
4. Prescribed by or in consultation with a pediatric/adult neurologist or a physician who is an expert in the treatment of DMD and other neuromuscular disorders
5. Prescriber understands that continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials (PI)
6. Patient's kidney function will be evaluated before and during treatment as required by the medication label
7. Weight must be received for drugs that have weight-based dosing
8. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Lexi-Drugs, national guidelines, or other peer-reviewed evidence
9. Patient will not use golodirsen (Vyondys 53<sup>®</sup>) together with viltolarsen (Viltepso<sup>®</sup>)

10. For Elevidys:

- a. Patient is 4-5 years old
- b. Patient is ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent)
- c. Baseline anti-AAVrh74 antibody titers <1:400 as determined by a total binding antibody ELISA
- d. Elevidys will not be used in combination with exon-skipping therapies (casimersen, eteplirsen, golodirsen, viltolarsen)
- e. Treatment is one time only

**Continuation of therapy:**

1. Updated chart notes demonstrating positive clinical response to therapy (such as improvement and/or stabilization compared to baseline)
2. Prescribed by or in consultation with a pediatric/adult neurologist or a physician who is an expert in the treatment of DMD and other neuromuscular disorders
3. For dose increases, the member's weight must be received
4. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Lexi-Drugs, national guidelines, or other peer-reviewed evidence
5. Patient will not use golodirsen (Vyondys 53<sup>®</sup>) together with viltolarsen (Viltepso<sup>®</sup>)

References:

1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; September 2016.
2. Vyondys 53 [package insert]. Sarepta Therapeutics, Inc.; Cambridge, MA. March 2020.
3. Viltepso [package insert]. NS Pharma, Inc. Paramus, NJ 07652
4. Amondys 45 [package insert]. Sarepta Therapeutics, Inc; Cambridge MA. February 2021
5. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2019. URL: <http://www.clinicalpharmacology.com>. Updated periodically
6. Mendell JR, et al; Eteplirsen Study Group. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-647.
7. Lee JJA, Saito T et al. Direct Reprogramming of Human DMD Fibroblasts into Myotubes for In Vitro Evaluation of Antisense-Mediated Exon Skipping and Exons 45-55 Skipping Accomplished by Rescue of Dystrophin Expression. *Methods Mol Biol*. 2018; 1828: 141-150
8. Bushby K, Finkel R, Birnkrant DJ, Case LE, Clemens PR, Cripe L, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol*; 2010 Jan; 9(1):77-93.

NJ DURB Prior Authorization Denial Report - 2nd Quarter 2023 (April - June)

	FFS	Aetna	Amerigroup	Horizon	UHC	Wellcare
<b>Total # of Enrolled Beneficiaries</b>	<b>71,503</b>	<b>133,886</b>	<b>254,728</b>	<b>1,241,100</b>	<b>432,930</b>	<b>113,030</b>
<b>Total # of Pharmacy Claims Processed</b>	<b>420,738</b>	<b>511,026</b>	<b>1,101,703</b>	<b>3,895,135</b>	<b>1,076,399</b>	<b>439,217</b>
<b>Total # of Members Requesting Prior Authorization*</b>	<b>1,258</b>	<b>3,136</b>	<b>6,960</b>	<b>24,436</b>	<b>8,010</b>	<b>2,366</b>
<b>Total Prior Authorizations Requests Received**</b>	<b>3,061 (0.7%)</b>	<b>4,379 (0.9%)</b>	<b>9,370 (0.9%)</b>	<b>31,386 (0.8%)</b>	<b>10,417 (1.0%)</b>	<b>3,630 (0.8%)</b>
<b>Received Requests Denials</b>	<b>224 (7%)</b>	<b>1,705 (39%)</b>	<b>3,733 (40%)</b>	<b>11,297 (36%)</b>	<b>5,012 (48%)</b>	<b>1,285 (35%)</b>
<b>Without Non-formulary Denials</b>	<b>224 (7%)</b>	<b>400 (9%)</b>	<b>1,472 (16%)</b>	<b>4,128 (13%)</b>	<b>1,741 (17%)</b>	<b>279 (8%)</b>
<b>Percentage Breakdown of Denials***</b>						
Clinical Criteria Not Met	121 (54%)	380 (22%)	1,148 (31%)	3,880 (34%)	1,429 (29%)	278 (22%)
Excluded Benefit	103 (46%)	17 (1%)	289 (8%)	248 (2%)	312 (6%)	1 (0%)
Non-formulary	0 (0%)	1,305 (77%)	2,261 (61%)	7,169 (63%)	3,271 (65%)	1,006 (78%)
Other	0 (0%)	3 (0%)	35 (1%)	0 (0%)	0 (0%)	0 (0%)
<b>Denials by Therapeutic Drug Classification****</b>						
Antihyperlipidemics	4.5%	6.7%	3.0%	2.7%	3.7%	3.0%
Antidepressants	0.9%	1.0%	1.7%	1.5%	1.3%	0.6%
Antihypertensives	1.3%	1.0%	0.6%	0.5%	1.0%	0.6%
Antianxiety	1.8%	0.3%	0.2%	0.3%	0.0%	0.0%
Antidiabetics (oral and insulin)	4.0%	8.6%	13.1%	26.2%	23.3%	21.6%
Anticoagulants		0.1%	0.0%	0.2%	0.4%	0.2%
Thyroid agents		0.4%		0.3%	0.5%	0.2%
Ulcer Drugs/Antispasmodics/Anticholinergics	21.9%	2.1%	2.3%	1.5%	2.5%	0.5%
ADHD/Anti-Narcolepsy/AntiObesity/Anorexiant		8.5%	7.7%	3.8%	2.6%	7.3%
Antipsychotic/Antimanic agents	9.4%	1.3%	1.7%	2.9%	1.3%	1.8%
Antiasthmatic and Bronchodilator agents	4.9%	7.3%	3.1%	6.0%	8.5%	2.2%
Antivirals (includes both HIV and Hep C)		0.5%	0.5%	0.4%	0.6%	1.6%
Digestive Aids (Digestive Enzymes)		0.2%	0.1%	0.1%	0.1%	0.3%
Anticonvulsants	1.8%	3.7%	1.1%	1.5%	1.8%	2.1%
Migraine Products	0.4%	2.2%	3.7%	4.2%	4.6%	4.2%
Analgesics Anti-inflammatory	5.4%	3.3%	2.7%	1.5%	1.4%	2.7%
Analgesic Opioids	0.9%	5.3%	4.9%	1.6%	2.2%	3.4%
Endocrine and Metabolic Agents-Misc (Growth Hormone)		1.3%	2.0%	0.8%	1.4%	1.8%
Psychotherapeutic And Neurological Agents - Misc (Multiple Sclerosis agents)		1.2%	0.8%	0.8%	0.4%	0.9%
Respiratory Agents-Misc (Cystic Fibrosis Agent – Combinations)		0.1%	0.1%	0.0%	0.0%	0.1%
Dermatologics (Antipsoriatics-Systemic)		15.8%	16.9%	12.7%	10.4%	11.1%

\* Value represents unduplicated data and will not include a member more than once, even if multiple requests are made.

\*\* Denominator for percentage is Total Number of Pharmacy Claims Processed.

\*\*\* See below for explanation of categories:

Clinical Criteria Not Met: includes categories such as Clinical Criteria Not Met, Drug-Drug Interaction, Therapeutic Duplication, Unacceptable Diagnosis

Excluded Benefit: includes categories such as Duration Exceeded, Excessive Dose, Mandatory Generic

Non-Formulary: includes categories such as Non-Formulary

Other: includes categories such as Directed Intervention, Multiple Pharmacies, Multiple Prescribers, Other DUR related rejections

\*\*\*\* Denominator contains total drug prior authorization requests denied. Breakdown of Therapeutic Drug Classification categories is a sample of prior authorization claims data and is not inclusive of all drug classes. Denial percentages will not equal one hundred percent.

Summary of DURB Recommendations

October 18, 2023

Meeting Date	Action Item	Status/DURB recommendation	Impact/Comments
July 2023	<p>Proposed protocol for Chimeric Antigen Receptor T-cell (CAR T-cell) products</p> <p>Proposed protocol for Qalsody (tofersen)</p> <p>Proposed addendum to the biologic receptor modifiers (BRMs) protocol for plaque psoriasis</p>	<ul style="list-style-type: none"> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol</li> <li>- The Board tabled the protocol pending consult with a dermatologist</li> </ul>	
April 2023	<p>Proposed protocol for Skysona® (elivaldogene autotemcel)</p> <p>Proposed protocol for Zynteglo® (betibeglogene autotemcel)</p> <p>Proposed protocol for Hemgenix® (etranacogene dezaparvovec)</p> <p>Proposed protocol for Leqembi® (lecanemab-irnb)</p> <p>Proposed protocol for Livmarli® (maralixibat)</p>	<ul style="list-style-type: none"> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol with a suggestion to change criterion #5 to read: Medication is prescribed by or in consultation with a hepatologist, gastroenterologist, or other specialist with experience in the treatment of the disease</li> </ul>	<p>The updated information was presented at the next meeting</p>
January 2023	<p>Addendum to Spinraza®/Zolgensma® protocols</p> <p>Addendum to Imcivree® (setmelanotide) protocol</p> <p>Addendum to Dupixent® protocol (atopic dermatitis)</p> <p>Proposed protocol for Gattex® (teduglutide)</p>	<ul style="list-style-type: none"> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol with suggestion to remove the word "adult" in the background section.</li> </ul>	<p>The updated information was presented at the next meeting</p>
October 2022	<p>Addendum to calcitonin gene-related peptide (CGRP) receptor antagonist protocol</p> <p>Proposed protocol for glucagon-like peptide-1 receptor agonists for T2D</p>	<ul style="list-style-type: none"> <li>- The Board recommended the protocol</li> <li>- The Board recommended the protocol with suggestions to reword criterion #3 and removal of criterion #1 under continuation of therapy</li> <li>- The Board recommended the protocol</li> </ul>	<p>An updated version was presented at the next meeting</p>

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Summary of DURB Recommendations

Meeting Date	Action Item	Status/DURB recommendation	Impact/Comments
	<p>Proposed protocol for biologics in moderate to severe asthma treatment</p> <p>Proposed protocol for Cholbam (cholic acid)</p> <p>Proposed protocol for Crysvita (burosumab-twza)</p>	<ul style="list-style-type: none"> <li>- The Board recommended the protocol with suggestion to remove the monitoring requirement in the "continuation of therapy" section</li> <li>- The Board recommended the protocol</li> </ul>	<p>An updated version was presented at the next meeting</p>