BlueCross BlueShield Arizona

An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINE

ATTRUBY™ (acoramidis) oral
TEGSEDI™ (inotersen) subcutaneous injection
VYNDAMAX™ (tafamidis) oral
VYNDAQEL® (tafamidis meglumine) oral
WAINUA™ (eplontersen) subcutaneous injection
Generic Equivalent (if available)

This Pharmacy Coverage Guideline (PCG):
- Provides information about the reasons, basis, and information sources we use for coverage decisions
- Is not an opinion that a drug (collectively "Service") is clinically appropriate or inappropriate for a patient
- Is not a substitute for a provider's judgment (Provider and patient are responsible for all decisions about appropriateness of care)
- Is subject to all provisions e.g. (benefit coverage, limits, and exclusions) in the member's benefit plan; and
- Is subject to change as new information becomes available.

Scope
- This PCG applies to Commercial and Marketplace plans
- This PCG does not apply to the Federal Employee Program, Medicare Advantage, Medicaid or members of out-of-state Blue Cross and/or Blue Shield Plans

Instructions & Guidance
- To determine whether a member is eligible for the Service, read the entire PCG.
- This PCG is used for FDA approved indications including, but not limited to, a diagnosis and/or treatment with dosing, frequency, and duration.
- Use of a drug outside the FDA approved guidelines, refer to the appropriate Off-Label Use policy.
- The "Criteria" section outlines the factors and information we use to decide if the Service is medically necessary as defined in the Member's benefit plan.
- The "Description" section describes the Service.
- The "Definition" section defines certain words, terms or items within the policy and may include tables and charts.
- The "Resources" section lists the information and materials we considered in developing this PCG
- **We do not accept patient use of samples as evidence of an initial course of treatment, justification for continuation of therapy, or evidence of adequate trial and failure.**
- Information about medications that require prior authorization is available at www.azblue.com/pharmacy. You must fully complete the request form and provide chart notes, lab workup and any other supporting documentation. The prescribing provider must sign the form. Fax the form to BCBSAZ Pharmacy Management at (602) 864-3126 or email it to Pharmacyprecert@azblue.com.

Criteria:

TEGSEDI (inotersen)
WAINUA (eplontersen)

- **Criteria for initial therapy:** Tegsedi (inotersen), Wainua (eplontersen), and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met:

  1. Prescriber is a physician specializing in the patient's diagnosis or is in consultation with a Neurologist

2. Individual is 18 years of age or older

3. Individual has a confirmed diagnosis of **polyneuropathy of hereditary transthyretin (hTTR)-mediated amyloidosis** and **ALL** of the following:
    a. Diagnosis confirmed by biopsy **or** genetic testing documenting pathogenic TTR mutation
    b. Signs and symptoms of polyneuropathy
    c. **ONE** of the following:
        i. Polyneuropathy disability (PND) stage III B or lower
        ii. Familial amyloid polyneuropathy (FAP) stage I or II
        iii. Neuropathy impairment score (NIS) is between 10 and 130

4. Individual has tried or is currently receiving or has documented failure, contraindication per FDA label, intolerance, or is not a candidate for at least **ONE** systemic agent for symptoms of polyneuropathy from **ONE** of the following pharmacologic classes:
    a. A gabapentin-type product (e.g., gabapentin [Neurontin], Lyrica [pregabalin])
    b. Duloxetine or venlafaxine
    c. A tricyclic antidepressant (e.g., amitriptyline, nortriptyline)

5. **If available:** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

6. **For Tegsedi only:** Individual has received and completed **ALL** the following **baseline tests** before initiation of treatment and with continued monitoring of the individual as clinically appropriate:
    a. Platelet count is at least 100 x 10^9/L [Note: This is waved if it is verified that Provider, Patient, and Pharmacy are enrolled in the REMS]
    b. Serum creatinine [Note: This is waved if it is verified that Provider, Patient, and Pharmacy are enrolled in the REMS]
    c. Estimated glomerular filtration rate (eGFR) is at least 45 mL/min/1.73 m^2 [Note: This is waved if it is verified that Provider, Patient, and Pharmacy are enrolled in the REMS]
    d. Urinary protein to creatinine ratio (UPCR) < 1,000 mg/g [Note: This is waved if it is verified that Provider, Patient, and Pharmacy are enrolled in the REMS]
    e. Urinalysis [Note: This is waved if it is verified that Provider, Patient, and Pharmacy are enrolled in the REMS]
    f. Alanine aminotransferase (ALT), aspartate aminotransferase (AST), and total bilirubin

7. **For Tegsedi only:** There are **NO** FDA-label contraindications such as:
    a. Platelet count < 100 x 10^9/L
    b. History of glomerulonephritis caused by Tegsedi
    c. History of hypersensitivity reaction to Tegsedi

8. Individual does not have moderate or severe hepatic impairment

9. Individual does not have a liver transplant

10. Individual does not have severe renal impairment or end-stage renal disease

11. Neuropathy is not due to other causes such as from diabetes mellitus, chronic alcohol, vitamin B12 deficiency, chronic inflammatory demyelinating polyneuropathy

12. Will not be used with Vyndaqel (tafamidis meglumine) or Vyndamax (tafamidis) or Onpattro (patisiran) or Amvuttra (vutrisiran), or Attruby (acoramidis)

Initial approval duration: 6 months

- **Criteria for continuation of coverage (renewal request):** Tegsedi (inotersen), Wainua (eplontersen), and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met **(samples are not considered for continuation of therapy)**:

1. Individual continues to be seen by a physician specializing in the patient's diagnosis or is in consultation with a Neurologist

2. Individual's condition has responded while on therapy with response defined as achieved and maintains at least a 25% improvement in: [Documentation from the medical record must be provided]
    a. Neurologic function (cranial nerves, reflexes, sensations),
    b. Motor function (muscle strength),
    c. Cardiac function (heart rate response to deep breathing, postural blood pressure),
    d. Quantitative sensory testing (touch-pressure and heat-pain)
    e. Peripheral nerve electrophysiology

3. Individual has been adherent with the medication

4. **If available:** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

5. Individual has not developed any contraindications or other significant adverse drug effects that may exclude continued use as follows:
    a. Contraindications as listed in the criteria for initial therapy section
    b. **For Tegsedi only:** Significant adverse effect such as:
        i. Thrombocytopenia
        ii. Glomerulonephritis
        iii. Nephrotic syndrome
        iv. Stroke
        v. Carotid Artery dissection

vi. Cytokine release symptoms
vii. Hepatic dysfunction or injury
viii. Detection of treatment emergent anti-platelet IgG antibodies

6. Will not be used with Vyndaqel (tafamidis meglumine) or Vyndamax (tafamidis) or Onpattro (patisiran) or Amvuttra (vutrisiran), or Attruby (acoramidis)

7. Individual does not have severe renal impairment or end-stage renal disease

8. Individual does not have a liver transplant

9. Individual does not have moderate or severe hepatic impairment

Renewal duration: 12 months

- Criteria for a request for non-FDA use or indication, treatment with dosing, frequency, or duration outside the FDA-approved dosing, frequency, and duration, refer to one of the following Pharmacy Coverage Guideline:

1. **Off-Label Use of Non-Cancer Medications**

2. **Off-Label Use of Cancer Medications**

ATTRUBY (acoramidis)
VYNDAMAX (tafamidis)
VYNDAQEL (tafamidis meglumine)

- **Criteria for initial therapy:** Attruby (acoramidis), Vyndamax (tafamidis), or Vyndaqel (tafamidis meglumine) and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met:

1. Prescriber is a physician specializing in the patient's diagnosis or is in consultation with a Cardiologist

2. Individual is 18 years of age or older

3. Individual has a confirmed diagnosis of symptomatic **cardiomyopathy** due to wild-type or hereditary **transthyretin (TTR)-mediated amyloid** (ATTR-CM) and **ALL** of the following:
   a. Has a history of at least **one** prior hospitalization for heart failure **or** clinical evidence of heart failure (without hospitalization) requiring diuretics
   b. Presence of amyloid deposits in tissue biopsy **or** technetium-based pyrophosphate radionuclide scintigraphy imaging (e.g., ^{99m}Tc-PYP, ^{99m}Tc-DPD, ^{99m}Tc-HMDP)

c. There is evidence of amyloid cardiac involvement by echocardiography or cardiac magnetic resonance imaging (e.g., end-diastolic interventricular septal wall thickness of > 12 mm or increased thickness of ventricular wall)

4. **ONE** of the following:
   a. **For wild-type ATTR-CM:** immunohistochemical analysis, scintigraphy, or mass spectrometry confirming presence of transthyretin precursor proteins
   b. **For hereditary ATTR-CM:** genetic testing confirming TTR gene mutation is pathogenic or likely pathogenic variant

5. **If available:** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

6. Individual has received and completed **ALL** the following **baseline tests** before initiation of treatment and with continued monitoring of the individual as clinically appropriate:
   a. 6-minute walk test is **ONE** of the following:
      i. > 100 meters for Vyndamax (tafamidis), or Vyndaqel (tafamidis meglumine)
      ii. ≥ 150 meters for Attruby (acoramidis)
   b. Plasma NT-proBNP is **ONE** of the following:
      i. ≥ 600 pg/mL for Vyndamax (tafamidis), or Vyndaqel (tafamidis meglumine)
      ii. ≥ 300 pg/mL for Attruby (acoramidis)

7. Individual does not have **ANY** of the following:
   a. New York Heart Association (NYHA) Class IV heart failure
   b. Heart failure due to other causes other than ATTR
   c. Light chain amyloidosis
   d. Implanted cardiac device
   e. Liver or heart transplantation

8. **ONE** of the following:
   a. **For Vyndamax (tafamidis) or Vyndaqel (tafamidis meglumine)**: Individual does not have severe hepatic impairment (Child-Pugh Class C)
   b. **For Attruby (acoramidis)**: Individual does not have hepatic impairment

9. **For Attruby (acoramidis) only**: Individual is not concurrently using UGT inducers and strong CYP3A inducers (e.g., rifampicin, phenytoin, phenobarbital, carbamazepine, others)

10. Individual does not use verapamil, diltiazem, doxycycline, or taurourodeoxycholic acid product

11. Will not be used with Tegsedi (inotersen) or Wainua (eplontersen) or Onpattro (patisiran) or Amvuttra (vutrisiran)

12. Attruby (acoramidis), Vyndamax (tafamidis) and Vyndaqel (tafamidis meglumine) will not be used concurrently or interchangeably

Initial approval duration: 6 months

- **Criteria for continuation of coverage (renewal request):** Attruby (acoramidis), Vyndamax (tafamidis), or Vyndaqel (tafamidis meglumine) and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met **(samples are not considered for continuation of therapy)**:

1. Individual continues to be seen by a physician specializing in the patient's diagnosis or is in consultation with a Cardiologist

2. Individual's condition has responded while on therapy with response defined as achieved and maintains **TWO** of the following: [Documentation from the medical record must be provided]
    a. No worsening or improvement in 6-minute walk test from baseline
    b. No worsening or improvement in NYHA functional class from baseline
    c. Reduction in plasma N-terminal B-type natriuretic peptide (NT-proBNP) from baseline
    d. At least a 30% reduction in non-elective cardiovascular related hospitalization

3. Individual has been adherent with the medication

4. **If available:** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

5. Individual has not developed any significant adverse drug effects that may exclude continued use

6. Individual does not have **ANY** of the following:
    a. New York Heart Association (NYHA) Class IV heart failure
    b. Heart failure due to other causes other than ATTR
    c. Light chain amyloidosis
    d. Implanted cardiac device
    e. Liver or heart transplantation

7. **ONE** of the following
    a. **For Vyndamax (tafamidis) or Vyndaqel (tafamidis meglumine)**: Individual does not have severe hepatic impairment (Child-Pugh Class C)
    b. **For Attruby (acoramidis)**: Individual does not have hepatic impairment

8. **For Attruby (acoramidis) only**: Individual is not concurrently using UGT inducers and strong CYP3A inducers (e.g., rifampicin, phenytoin, phenobarbital, carbamazepine, others)

9. Individual does not use verapamil, diltiazem, doxycycline, or taurourodeoxycholic acid product

10. Will not be used with Tegsedi (inotersen) or Wainua (eplontersen) or Onpattro (patisiran) or Amvuttra (vutrisiran)

11. Attruby (acoramidis), Vyndamax (tafamidis) and Vyndaqel (tafamidis meglumine) will not be used concurrently or interchangeably

Renewal duration: 12 months

- Criteria for a request for non-FDA use or indication, treatment with dosing, frequency, or duration outside the FDA-approved dosing, frequency, and duration, refer to one of the following Pharmacy Coverage Guideline:

1. **Off-Label Use of Non-Cancer Medications**

2. **Off-Label Use of Cancer Medications**

Description:

Tegsedi (inotersen) is indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) in adults.

Tegsedi (inotersen) is an antisense oligonucleotide (ASO) that causes degradation of mutant and wild-type TTR mRNA through binding to the TTR mRNA, which results in a reduction of serum TTR protein and TTR protein deposits in tissues. By interfering with the RNA that carries damaged sequences for hATTR amyloidosis, it prevents the formation of amyloid fibrils. Because of the risks of serious bleeding caused by severe thrombocytopenia and because of glomerulonephritis, both of which require frequent monitoring, Patients using Tegsedi (inotersen) will need frequent testing for platelet counts and kidney function before, during and after treatment. Tegsedi (inotersen) is available only through a restricted distribution program under a Risk Evaluation and Mitigation Strategy (REMS) called the TEGSEDI REMS Program.

Wainua (eplontersen) subcutaneous injection is a transthyretin-directed antisense oligonucleotide indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults. Eplontersen is an antisense oligonucleotide-GalNAc conjugate that causes degradation of mutant and wild-type TTR mRNA through binding to the TTR mRNA, which results in a reduction of serum TTR protein and TTR protein deposits in tissues.

Vyndamax (tafamidis) or Vyndaqel (tafamidis meglumine) is indicated for the treatment of the cardiomyopathy of wild type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular mortality and cardiovascular-related hospitalization.

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Generic Equivalent (if available)

The active ingredient of both Vyndamax (tafamidis) and Vyndaqel (tafamidis meglumine) is tafamidis. Tafamidis is a TTR stabilizer that selectively binds to TTR at the thyroxine binding sites and stabilizes the tetramer of the TTR transport protein, slowing dissociation into monomers that is the rate-limiting step in the amyloidogenic process. Tafamidis stabilizes both wild-type TTR tetramers and the tetramers of 14 TTR variants when tested clinically as well as 25 TTR variants tested ex vivo. Tafamidis is an analog of diflunisal that does not have anti-inflammatory properties.

Attruby (acoramidis) is indicated for the treatment of the cardiomyopathy of wild-type or variant transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular death and cardiovascular-related hospitalization. Acoramidis is a selective stabilizer of transthyretin (TTR). Acoramidis binds TTR at thyroxine binding sites and slows dissociation of the TTR tetramer into its constituent monomers, the rate-limiting step in amyloidogenesis.

Transthyretin amyloidosis is a slowly progressive condition characterized by the buildup of abnormal deposits of amyloid (amyloidosis) in organs and tissues. Protein deposition most frequently occurs in the peripheral nervous system resulting in a loss of sensation in the extremities (peripheral neuropathy). The autonomic nervous system may also be affected by amyloidosis. In some cases, the brain and spinal cord are affected. Other areas of amyloidosis include the heart, kidneys, eyes, and gastrointestinal tract. The age at which symptoms begin to develop varies widely among individuals with this condition and is typically between ages 20 and 70. The condition is inherited in an autosomal dominant pattern. The disease is caused by a mutation of the *TTR* gene located on chromosome 18 where valine is replaced by methionine at position 30 (TTR V30M or Val30Met). There are more than 130 mutations described.

The forms of transthyretin amyloidosis are distinguished by symptoms and the body system affected. The neuropathic form primarily affects the peripheral and autonomic nervous systems, resulting in peripheral neuropathy and difficulty controlling bodily functions. Impairments in bodily functions can include sexual impotence, diarrhea, constipation, problems with urination, and orthostatic hypotension. Some experience heart and kidney problems as well. Various eye problems may occur, such as cloudiness of the clear gel that fills the eyeball (vitreous opacity), dry eyes, glaucoma, or pupils with an irregular or "scalloped" appearance. Some also develop carpal tunnel syndrome, characterized by numbness, tingling, and weakness in the hands and fingers.

The leptomeningeal form primarily affects the central nervous system. In this form, amyloidosis occurs in the leptomeninges. Protein buildup can cause strokes and hemorrhage, hydrocephalus, ataxia, muscle stiffness and weakness (spastic paralysis), seizures, and loss of intellectual function. Eye problems similar to those in the neuropathic form may also occur.

The cardiac form primarily affects the heart which can lead to arrhythmias, cardiomegaly, or orthostatic hypertension. These abnormalities can lead to progressive heart failure and death. Occasionally, people with the cardiac form of transthyretin amyloidosis have mild peripheral neuropathy. The cardiac form can be hereditary or non-hereditary. The non-hereditary form is caused by aggregation of the wild-type transthyretin protein and is also known as Senile Systemic Amyloidosis.

Various stages of disease have been described. Patients with stage 0 disease are usually asymptomatic but have both a variant form of the TTR gene and evidence of amyloid deposits. Patients with stage I (mild) disease are still ambulatory, patients with stage II (moderate) disease are ambulatory but require assistance, and patients with stage III (severe) disease are bedridden or wheelchair bound.

Tissue biopsy should be used to confirm the diagnosis in all cases of amyloidosis, although the diagnosis of amyloidosis may be suspected on the basis of history and clinical manifestations. Tissue biopsy is done using Congo red staining and an immune histochemical study with anti-TTR antibodies. Genetic testing is needed to document the pathogenic mutation. If it is normal, TTR-FAP is excluded. Current techniques for performing sequence analysis of *TTR*, the only gene known to be associated with TTR amyloidosis, detect >99% of disease-causing mutations.

Orthotopic liver transplantation (OLTX), which removes the main production site of the amyloidogenic protein, has historically been the standard of care for hereditary TTR amyloidosis. OLTX is not effective in the non-neuropathic forms of familial TTR amyloidosis (i.e., cardiac amyloidosis, leptomeningeal amyloidosis, and familial oculoleptomeningeal amyloidosis [FOLMA]).

There are two other modes of treatment. The first is to reduce or halt the amount of mutant transthyretin that is synthesized through gene silencing by the liver. This approach employs use of small interfering RNA (patisiran) and antisense oligonucleotides (inotersen). The other approach is to stabilize mutant tetramers of transthyretin to prevent amyloidogenic monomers. Tetramer stabilizers include diflunisal and tafamidis. Currently under investigation is use of agent(s) to degrade amyloid fibrils that have already been deposited in tissues.

About 3,000 Americans are believed to have polyneuropathy caused by hATTR, which results from abnormally bent and folded proteins produced by mutated RNA. The amyloid fibrils (unusable proteins) deposit in nerves, where they produce pain in the arms, feet, hands, and legs. Because they also accumulate in organ tissue, they can enlarge the heart and damage other organs.

Definitions:

U.S. Food and Drug Administration (FDA) MedWatch Forms for FDA Safety Reporting
MedWatch Forms for FDA Safety Reporting | FDA

Risk Evaluation and Mitigation Strategy (REMS) Program:
Use of Tegsedi (inotersen) is subject to a Risk Evaluation and Mitigation Strategies (REMS) program that requires provider, patient, and dispensing pharmacy be enrolled into the program. Only providers and Pharmacies enrolled into the REMS may prescribe and dispense the drug, respectively, to individuals who are also in the program. A REMS program attempts to manage known or potentially serious risks associated with a drug product and is required by the Food and Drug Administration (FDA) for some drugs to ensure that the benefits of a drug outweigh its risks.

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Because of the risks of serious bleeding caused by severe thrombocytopenia and because of glomerulonephritis, both of which require frequent monitoring, Tegsedi (inotersen) is available through a restricted REMS program

Requirements of the Tegsedi (inotersen) REMS Program include the following:

- Prescribers must be certified within the program by enrolling and completing training
- Patients must enroll in the program and comply with ongoing monitoring requirements
- Pharmacies must be certified with the program and must only dispense to patients who are authorized to receive TEGSEDI.

Other names for polyneuropathy amyloidosis:

Familial amyloid polyneuropathy type I (Portuguese-Swedish-Japanese type)
Familial amyloid polyneuropathy type II (Indiana/Swiss or Maryland/German type)
Familial TTR amyloidosis
Amyloid transthyretin polyneuropathy (ATTR-PN)
Familial amyloidotic polyneuropathy (FAP)

Staging:

Clinical staging of TTR-FAP	
Stage 0	No symptoms
Stage I	Unimpaired ambulation; mostly mild sensory, motor, and autonomic neuropathy in the lower limbs
Stage II	Assistance with ambulation required; mostly moderate impairment progression to the lower limbs, upper limbs, and trunk
Stage III	Wheelchair-bound or bedridden; severe sensory, motor, and autonomic involvement of all limbs

Polyneuropathy Disability Staging:

Polyneuropathy Disability Stage	
Stage	**Description**
0	No symptoms
I	Sensory disturbances but preserved walking capability
II	Impaired walking capacity but ability to walk without a stick or crutches
IIIA	Walking with the help of one stick or crutch
IIIB	Walking with the help of two sticks or crutches
IV	Confined to a wheelchair or bedridden

Vitamin A:

Vitamin A (retinol, retinoic acid) is a nutrient important to vision, growth, cell division, reproduction and immunity. Vitamin A is found in many foods, such as spinach, dairy products and liver. Other sources are foods rich in beta-carotene, such as green leafy vegetables, carrots and cantaloupe. Your body converts beta-carotene into vitamin A. The recommended daily amount of vitamin A is 900 micrograms (mcg) for adult men and 700 mcg for adult women.

https://ods.od.nih.gov/factsheets/VitaminA-Consumer/
Currently, vitamin A is listed on food and supplement labels in international units (IUs) even though nutrition scientists rarely use this measure. Conversion rates between mcg retinol activity equivalents (RAE) and IU are as follows:

- 1 IU retinol = 0.3 mcg RAE
- 1 IU beta-carotene from dietary supplements = 0.15 mcg RAE
- 1 IU beta-carotene from food = 0.05 mcg RAE
- 1 IU alpha-carotene or beta-cryptoxanthin = 0.025 mcg RAE

Resources:

Attruby (acoramidis) tabs product information, revised by BeidgeBio Pharma, Inc 11-2024. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed December 25, 2024.

Tegsedi (inotersen) subcutaneous injection product information, revised by Akcea Therapeutics, Inc. 01-2024. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed June 11, 2024.

Wainua (eplontersen) subcutaneous injection product information, revised by AstraZeneca Pharmaceuticals, LP. 12-2023. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed June 11, 2024.

Vyndaqel (tafamidis meglumine) and Vyndamax (tafamidis) caps product information, revised by Pfizer Laboratories Div Pfizer Inc 10-2023. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed June 11, 2024.

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