Texas Prior Authorization Program
Clinical Criteria

Drug/Drug Class

Niemann-Pick Disease Type C Agents

Clinical Criteria Information Included in this Document

Aqneursa (Levacetylleucine)

- [Drugs requiring prior authorization:](#) the list of drugs requiring prior authorization for this clinical criteria

- [Prior authorization criteria logic:](#) a description of how the prior authorization request will be evaluated against the clinical criteria rules

- [Logic diagram:](#) a visual depiction of the clinical criteria logic

- [Supporting tables:](#) a collection of information associated with the steps within the criteria (diagnosis codes, procedure codes, and therapy codes); provided when applicable

- [References](#): clinical publications and sources relevant to this clinical criteria

Miplyffa (Arimoclomol Citrate)

- [Drugs requiring prior authorization:](#) the list of drugs requiring prior authorization for this clinical criteria

- [Prior authorization criteria logic:](#) a description of how the prior authorization request will be evaluated against the clinical criteria rules

- [Logic diagram:](#) a visual depiction of the clinical criteria logic

- [Supporting tables:](#) a collection of information associated with the steps within the criteria (diagnosis codes, procedure codes, and therapy codes); provided when applicable

- [References](#): clinical publications and sources relevant to this clinical criteria

Note: Click the hyperlink to navigate directly to that section.

Revision Notes
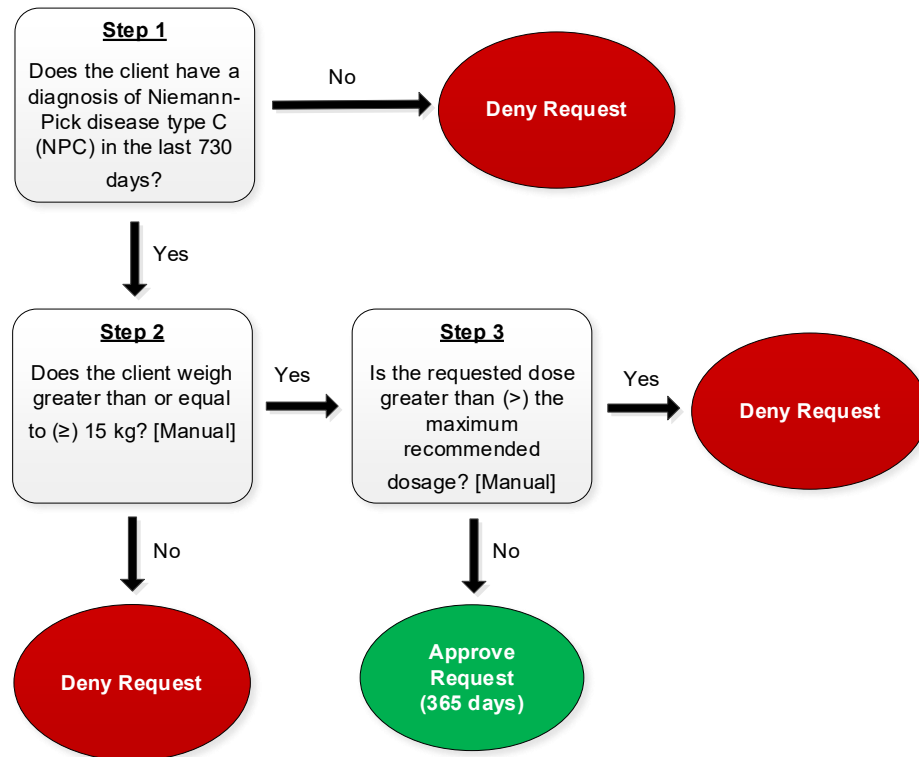
Initial publication and presentation to the DUR Board

PAXPRESS™	**Aqneursa (Levacetylleucine)**
	Drugs Requiring Prior Authorization

The listed GCNs may not be an indication of Texas Medicaid Formulary coverage. To learn the current formulary coverage, visit txvendordrug.com/formulary/formulary-search.

Drugs Requiring Prior Authorization	
Label Name	**GCN**
AQNEURSA 1 GRAM GRANULE PACKET	56268

PAXPRESS™

Aqneursa (Levacetylleucine)

Clinical Criteria Logic

1. Does the client have a [diagnosis of Niemann-Pick disease type C (NPC)](#) in the last 730 days?

   [ ] Yes – Go to #2

   [ ] No – Deny

2. Does the client weigh greater than or equal to (≥) 15 kg? [Manual]

   [ ] Yes – Go to #3

   [ ] No – Deny

3. Is the requested dose greater than (>) the [maximum recommended dosage](#)? [Manual]

   [ ] Yes – Deny

   [ ] No – Approve (365 days)

PA_XPRESS_™

Aqneursa (Levacetylleucine)
Clinical Criteria Logic Diagram

Step 1

Does the client have a diagnosis of Niemann-Pick disease type C (NPC) in the last 730 days?

→ **No** → **Deny Request**

↓ **Yes**

Step 2

Does the client weigh greater than or equal to (≥) 15 kg? [Manual]

→ **Yes** →

Step 3

Is the requested dose greater than (>) the maximum recommended dosage? [Manual]

→ **Yes** → **Deny Request**

↓ **No** (Step 2)

Deny Request

↓ **No** (Step 3)

Approve Request (365 days)

Aqneursa (Levacetylleucine)

Clinical Criteria Supporting Tables

Table 1 (diagnosis of Niemann-Pick disease type C (NPC)) Required diagnosis: *1* Look back timeframe: *730* days	
ICD-10 Code	**Description**
E75242	NIEMANN-PICK DISEASE TYPE C

Table 3 (Aqneursa maximum recommended dosing)	
15 to <25 kg	2 grams/day
25 to <35 kg	3 grams/day
35 kg or more	4 grams/day

PA*XPRESS*™   **Miplyffa (Arimoclomol Citrate)**

Drugs Requiring Prior Authorization

The listed GCNs may not be an indication of Texas Medicaid Formulary coverage. To learn the current formulary coverage, visit txvendordrug.com/formulary/formulary-search.

Drugs Requiring Prior Authorization	
Label Name	**GCN**
MIPLYFFA 47 MG CAPSULE	56255
MIPLYFFA 62 MG CAPSULE	56256
MIPLYFFA 93 MG CAPSULE	56257
MIPLYFFA 124 MG CAPSULE	56258

PAXPRESS™	**Miplyffa (Arimoclomol Citrate)**
	Clinical Criteria Logic

1. Does the client have a diagnosis of Niemann-Pick disease type C (NPC) in the last 730 days?

    [ ] Yes – Go to #2

    [ ] No – Deny

2. Is the client greater than or equal to (≥) 2 years of age?

    [ ] Yes – Go to #3

    [ ] No – Deny

3. Will the client have concurrent therapy with miglustat?

    [ ] Yes – Go to #4

    [ ] No – Deny

4. Does the client have an eGFR ≥ 15 to < 50 mL/minute in the last 365 days?

    [ ] Yes – Go to #5

    [ ] No – Go to #6

5. Is the requested dose greater than (>) the maximum recommended adjusted dosage for the client's actual body weight? [Manual]

    [ ] Yes – Deny

    [ ] No – Approve (365 days)

6. Is the requested dose greater than (>) the maximum recommended dosage for the client's actual body weight? [Manual]

    [ ] Yes – Deny

    [ ] No – Approve (365 days)

PAXPRESS™

Miplyffa (Arimoclomol Citrate)

Clinical Criteria Logic Diagram

Step 1

Does the client have a diagnosis of Niemann-Pick disease type C (NPC) in the last 730 days?

— No → **Deny Request**

— Yes ↓

Step 2

Is the client greater than or equal to (≥) 2 years of age?

— Yes →

— No → **Deny Request**

Step 3

Will the client have concurrent therapy with miglustat?

— Yes →

— No → **Deny Request**

Step 4

Does the client have an eGFR ≥ 15 to < 50 mL/minute in the last 365 days?

— No →

— Yes ↓

Step 5

Is the requested dose greater than (>) the maximum recommended adjusted dosage for the client's actual body weight? [Manual]

— No → **Approve Request (365 days)**

— Yes → **Deny Request**

Step 6

Is the requested dose greater than (>) the maximum recommended dosage for the client's actual body weight? [Manual]

— Yes → **Deny Request**

— No → **Approve Request (365 days)**

PAXPRESS™
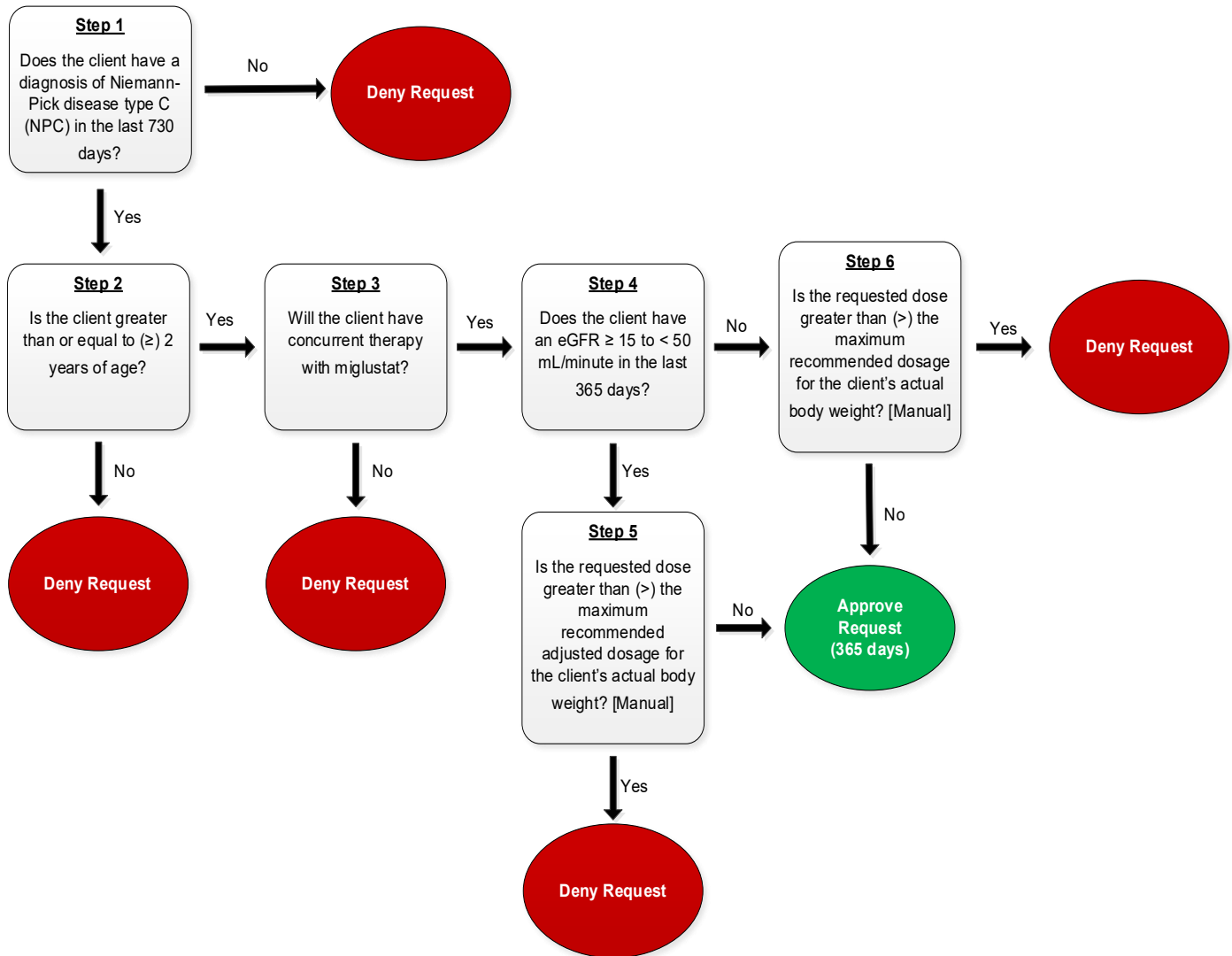
Miplyffa (Arimoclomol Citrate)
Clinical Criteria Supporting Tables

Table 1 (diagnosis of Niemann-Pick disease type C (NPC)) Required diagnosis: *1* Look back timeframe: *730 days*	
ICD-10 Code	**Description**
E75242	NIEMANN-PICK DISEASE TYPE C

Table 3 (miglustat) Required quantity: *1* Look back timeframe: *current therapy*	
GCN	**Label Name**
19453	MIGLUSTAT 100 MG CAPSULE
54801	OPFOLDA 65 MG CAPSULE
19453	YARGESA 100 MG CAPSULE
19453	ZAVESCA 100 MG CAPSULE

Table 4 (eGFR ≥ 15 to < 50 mL/minute) Required diagnosis: *1* Look back timeframe: *365 days*	
ICD-10 Code	**Description**
N183	CHRONIC KIDNEY DISEASE, STAGE 3 (MODERATE)
N184	CHRONIC KIDNEY DISEASE, STAGE 4 (SEVERE)

Table 5 (Miplyffa maximum recommended adjusted dosage)	
Actual Body Weight	**Dose Per Day**
8 kg to 15 kg	94 mg/day

Table 5 (Miplyffa maximum recommended adjusted dosage)	
> 15 kg to 30 kg	124 mg/day
> 30 kg to 55 kg	186 mg/day
> 55 kg	248 mg/day

Table 6 (Miplyffa maximum recommended dosage)	
Actual Body Weight	**Dose Per Day**
8 kg to 15 kg	141 mg/day
> 15 kg to 30 kg	186 mg/day
> 30 kg to 55 kg	279 mg/day
> 55 kg	372 mg/day

1. Clinical Pharmacology [online database]. Tampa, FL: Elsevier/Gold Standard, Inc.; 2025. Available at www.clinicalpharmacology.com. Accessed on July 25, 2025.

2. Micromedex [online database]. Available at www.micromedexsolutions.com. Accessed on July 25, 2025.

3. Aqneursa Prescribing Information. Austin, TX. IntraBio Inc. September 2024.

4. Miplyffa Prescribing Information. Celebration FL. Zevra Therapeutics Inc. September 2024.

5. 2025 ICD-10-CM Diagnosis Codes, Volume 1. 2025. Available at www.icd10data.com. Accessed on July 25, 2025.

Niemann-Pick Disease Type C Agents

Publication History

The Publication History records the publication iterations and revisions to this document. Notes for the *most current revision* are also provided in the Revision Notes on the first page of this document.

Publication Date	Notes
07/25/2025	• Initial publication and presentation to the DUR Board