# Gaining access to exon-skipping therapy

## Your guide to understanding the insurance process and considerations for exon-skipping therapy

**Finn**
Living with Duchenne

SAREPTA THERAPEUTICS

# What's inside?

**Exon-skipping therapies** use **phosphorodiamidate morpholino oligomers (PMOs)** modeled after the natural framework of RNA to "skip" over the missing parts of the gene (or exons) that cause errors in making dystrophin and lead to Duchenne.[1] Once you and your doctor decide to pursue exon-skipping therapy for Duchenne, getting approval from your insurance company to help cover the costs of the medication can require multiple steps.

**This guide can help you navigate the insurance process. It has information that can help you:**



SareptAssist is your partner to help manage the stresses of starting and staying on therapy. Reach out to us directly with any questions at 1-888-SAREPTA (1-888-727-3782) Monday through Friday, 8:30 AM - 6:30 PM ET.

# Understand your insurance

Health insurance can help pay for a portion of the costs of medical care and treatment. **The amount and types of costs covered vary according to the type of insurance you have.**[2]

## Types of insurance

**Private (commercial) and public (government) are the most common types of health insurance.**

### Private or commercial

**Private or commercial insurance can be offered through an employer or by purchasing it directly from an insurance company or state insurance exchange.**

Employers may offer a variety of plans during their **open enrollment period**, and each plan may vary in coverage, costs, and out-of-pocket expenses.[3]

**Knowing the type of plan your employer has can help you understand the type of coverage offered. Reach out to your employer's human resources department or refer to materials such as an employee handbook for more information on your plan.**

### Public or government-sponsored

**Public or government-sponsored plans are provided at the federal and state level for people who qualify for assistance.[2]**

Medicaid and Medicare are common types of public insurance that provide health coverage to millions of Americans.

Federal law requires states to provide Medicaid coverage to certain groups of individuals, including (but not limited to)[4]:

- Low-income families*
- Qualified pregnant women and children
- Individuals receiving Supplemental Security Income (SSI)

**Dual eligibility applies to individuals who receive medical coverage from both Medicare and Medicaid.[5]**

**Some patients may be eligible for Medicaid as secondary insurance, meaning that Medicaid will be the payer of last resort for costs not covered by their commercial insurance.[6]**

*Based on federal poverty guidelines published in January of each year. Note, guidelines vary by family size. In addition, there is one set of figures for the 48 contiguous states and Washington, DC; one set for Alaska; and one set for Hawaii.[7] https://aspe.hhs.gov/topics/poverty-economic-mobility/poverty-guidelines

# Medicaid and Medicare

People with Duchenne may be covered by a Medicaid or Medicare program. Below are important facts about these programs to help you know the differences. **Your SareptAssist Case Manager can share information with you about the different programs and how to enroll.**

MEDICAID

## Medicaid is a joint federal and state insurance program that covers millions of people, including[8]:

- Low-income households
- Adults
- Children
- Pregnant women
- Elderly adults
- People with disabilities

Medicaid is administered by states according to federal requirements. You may hear the terms **Medicaid fee-for-service or Medicaid Managed Care.** If you are enrolled in Medicaid fee-for-service, that means the state pays healthcare providers directly for health services. If you are enrolled in a Medicaid Managed Care program, that means you are part of a healthcare plan. In this case, the state pays the plan, and the plan then pays providers for services.[8,9]

All Medicaid programs provide coverage for prescription medications, typically at the lowest cost to beneficiaries.[9]

MEDICARE

## Medicare offers health insurance for people:

- Aged 65 years and older[10]
- Under age 65 who have certain disabilities[10]
- Of all ages with end-stage renal disease and amyotrophic lateral sclerosis (ALS)[11]

Within Medicare, there are different parts that cover different healthcare needs, such as[12]:

| PART A | PART B | PART C | PART D |
|---|---|---|---|
| Hospital stays | Doctor visits | Medicare Advantage plan* | Prescription drug costs |

Patients over the age of 20 with Duchenne qualify for Medicare after receiving **Social Security Disability Insurance (SSDI)** benefits for at least 2 years.[13] For more information about how to qualify for Medicare, visit SSA.gov.

*This is a managed care plan that offers both Medicare Parts A and B benefits and other supplemental benefits that may not be offered by traditional Medicare fee-for-service plans.

**There are also special federal and state programs that cover the costs associated with Duchenne. For example, in addition to Medicaid and Medicare, people may be covered by the Children's Health Insurance Program (CHIP) or may qualify for disability benefits. Look to page 5 for details.**

# Special federal and state programs

People with Duchenne may automatically qualify for one or more of these special federal or state programs that may **cover costs of drug infusions, transportation, hospital care, doctor visits, and home healthcare**. It's important to know about them and how to enroll. Your SareptAssist Case Manager can help.

## Supplemental Security Income or Social Security Disability

**Each state has different qualification requirements for Medicaid coverage.** Children with Duchenne may be eligible for Social Security Administration (SSA) benefits through the Supplemental Security Income or Social Security Disability Insurance programs—which means they may qualify for Medicaid.[4,14]

## Compassionate Allowances

The SSA Compassionate Allowances (CAL) program is available for adults with Duchenne. This program identifies severe medical diseases, including Duchenne, that automatically meet SSA standards for disability benefits, and **fast tracks the Medicaid application process**. Today, there are more than 250 conditions on the list.[16-18]

## Children's Health Insurance Program (CHIP)

CHIP is a joint federal-state program offering **free or low-cost coverage for uninsured children and teens under 19 years of age** in families with incomes too high to qualify for Medicaid.[15]

## Medicaid waiver or a Katie Beckett (available in some states)

When assessing types of Medicaid coverage for Duchenne, you may hear the term Medicaid waiver or a "Katie Beckett"—named for a young girl who required ongoing use of a ventilator at home. These are expanded programs that some states may offer to certain groups of people, based on different criteria in each state. **These waiver programs often provide coverage for families of children with exceptional health needs that may otherwise not qualify for Medicaid, and often help them remain home for health services.**[19,20]

**Visit Medicaid.gov to learn more about Medicaid programs in your state. You can also ask your SareptAssist Case Manager about which programs may be best for you.**

# How costs are covered

Whichever type of plan you have (eg, private or public), your insurance company **may share the costs of medications and services with you** by paying a portion of the costs, and you pay the out-of-pocket expenses, if any. The structure and amount of expenses are outlined below.

## Premium

Amount you pay to participate in the health plan, which will vary depending on the plan and type of coverage you select (eg, individual, couple, family). You may be responsible for these costs biweekly, monthly, quarterly, or annually.[21]

## Deductible

Set amount you pay each year before the plan covers a portion of the costs.[22]

## Co-insurance

Percentage of a medical charge you pay after you've received healthcare services. Typically, co-insurance is a fixed percentage, meaning you'll always be charged the same percentage (eg, 20%) of the total bill each time.[21,23]

## Co-pay

In addition to the deductible, you may be required to pay a co-pay—or specific dollar amount—for every doctor visit or medication. This amount will be less than the deductible and and is typically a fixed cost.[24]

The Affordable Care Act set a maximum limit for the amount health plan members have to pay in out-of-pocket expenses. This limit applies to your deductible, co-insurance, or co-pay. These limits vary by plan and type of health service (eg, in-network vs out-of-network).[25]

**How does your plan cover costs? Knowing the terms deductible, co-insurance, and co-pay can help you understand your out-of-pocket expenses for Duchenne treatment.**

# How does a deductible work?

**Member pays toward deductible**

**Deductible is met**

Member pays co-pay/ co-insurance toward out-of-pocket maximum.

**Out-of-pocket maximum met**

Health plan pays 100% of all medical expenses moving forward.

| To give you an example: | |
|---|---|
| Let's say your deductible is $2000 | $2000 |
| You go to the doctor with abdominal pain and pay $100 for the office visit | -$100 |
| You still have $1900 more to reach your deductible | $1900 |
| You pay for an X-ray your doctor orders | -$500 |
| You still have $1400 to reach your deductible | $1400 |
| Based on the X-ray, your doctor recommends surgery that costs $2000 You pay the $1400 left of your deductible | -$1400 |
| Your remaining deductible is now $0 | $0 |
| The health insurance plan pays the remaining $600 | $600 |
| Your doctor recommends a series of follow-ups and since you have met your deductible, you are only responsible for the co-pay amounts at your appointments (related or unrelated to an ongoing health issue) for the rest of the calendar year or plan year depending on your insurance | Pay co-pay amount |

Many plans pay for certain services, such as checkups or disease management programs, before you have met your deductible. Check your plan details.[21]

Deductible  Paid by you
Paid for by your health insurance plan

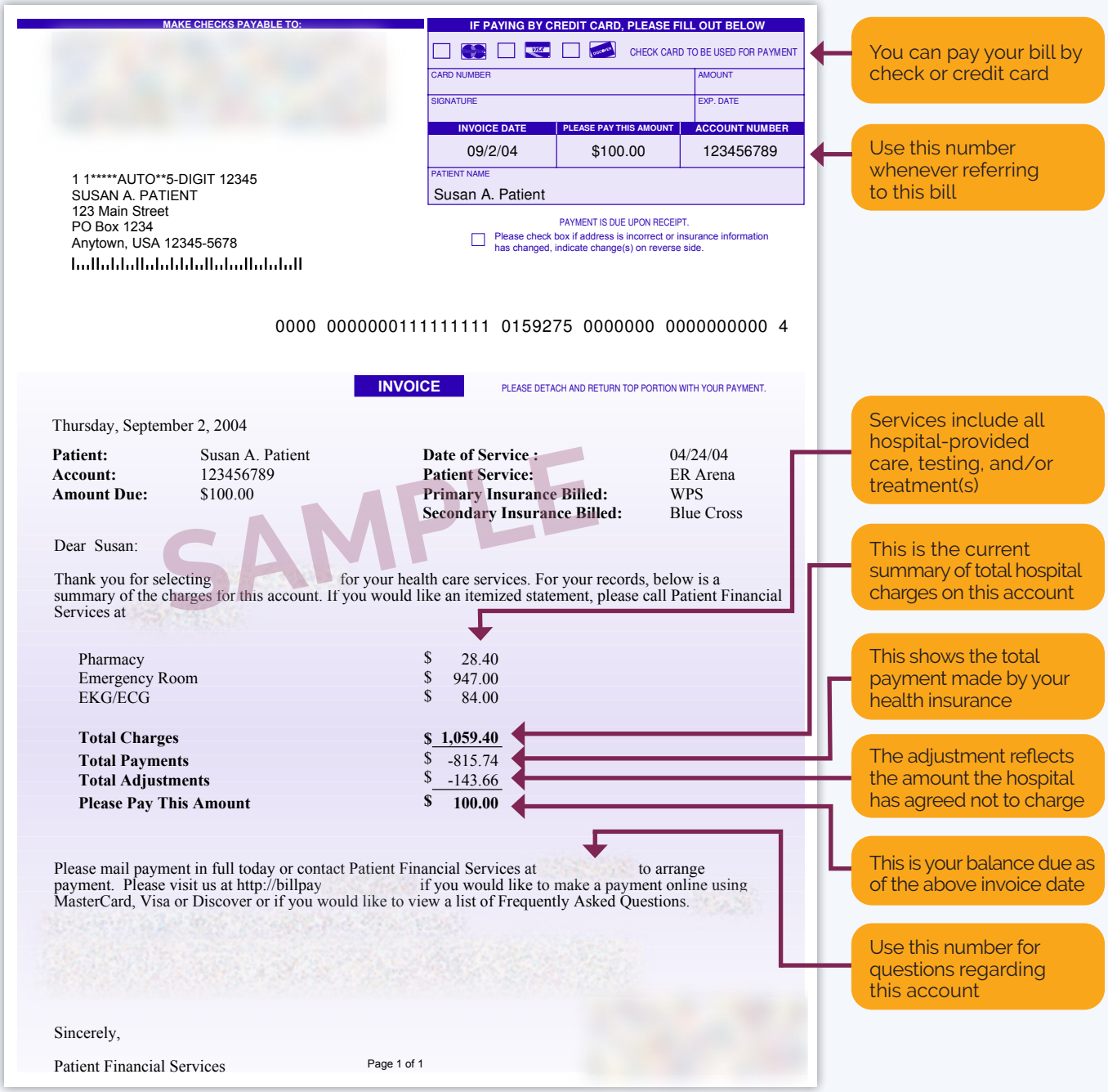
SAREPTA THERAPEUTICS

# Sample bills

You may receive an **explanation of benefits (EOB)** that appears to be a bill. An EOB is not a bill—it helps you understand what your health plan covers and what you'll need to pay when you do receive a bill.[21] If you have any questions, reach out to your SareptAssist Case Manager.

We have provided 2 sample bills with important information you should look out for. Note that your actual bill may look different from the examples provided below.

## Hospital outpatient service sample bill



MAKE CHECKS PAYABLE TO:

IF PAYING BY CREDIT CARD, PLEASE FILL OUT BELOW

CHECK CARD TO BE USED FOR PAYMENT

CARD NUMBER | AMOUNT

SIGNATURE | EXP. DATE

| INVOICE DATE | PLEASE PAY THIS AMOUNT | ACCOUNT NUMBER |
|---|---|---|
| 09/2/04 | $100.00 | 123456789 |

PATIENT NAME: Susan A. Patient

1 1*****AUTO**5-DIGIT 12345
SUSAN A. PATIENT
123 Main Street
PO Box 1234
Anytown, USA 12345-5678

PAYMENT IS DUE UPON RECEIPT.

Please check box if address is incorrect or insurance information has changed, indicate change(s) on reverse side.

0000 0000000111111111 0159275 0000000 0000000000 4

**INVOICE**

PLEASE DETACH AND RETURN TOP PORTION WITH YOUR PAYMENT.

Thursday, September 2, 2004

| | | | |
|---|---|---|---|
| **Patient:** | Susan A. Patient | **Date of Service :** | 04/24/04 |
| **Account:** | 123456789 | **Patient Service:** | ER Arena |
| **Amount Due:** | $100.00 | **Primary Insurance Billed:** | WPS |
| | | **Secondary Insurance Billed:** | Blue Cross |

SAMPLE

Dear Susan:

Thank you for selecting for your health care services. For your records, below is a summary of the charges for this account. If you would like an itemized statement, please call Patient Financial Services at

| | |
|---|---|
| Pharmacy | $ 28.40 |
| Emergency Room | $ 947.00 |
| EKG/ECG | $ 84.00 |
| **Total Charges** | **$ 1,059.40** |
| **Total Payments** | $ -815.74 |
| **Total Adjustments** | $ -143.66 |
| **Please Pay This Amount** | **$ 100.00** |

Please mail payment in full today or contact Patient Financial Services at to arrange payment. Please visit us at http://billpay if you would like to make a payment online using MasterCard, Visa or Discover or if you would like to view a list of Frequently Asked Questions.

Sincerely,

Patient Financial Services

Page 1 of 1

You can pay your bill by check or credit card

Use this number whenever referring to this bill

Services include all hospital-provided care, testing, and/or treatment(s)

This is the current summary of total hospital charges on this account

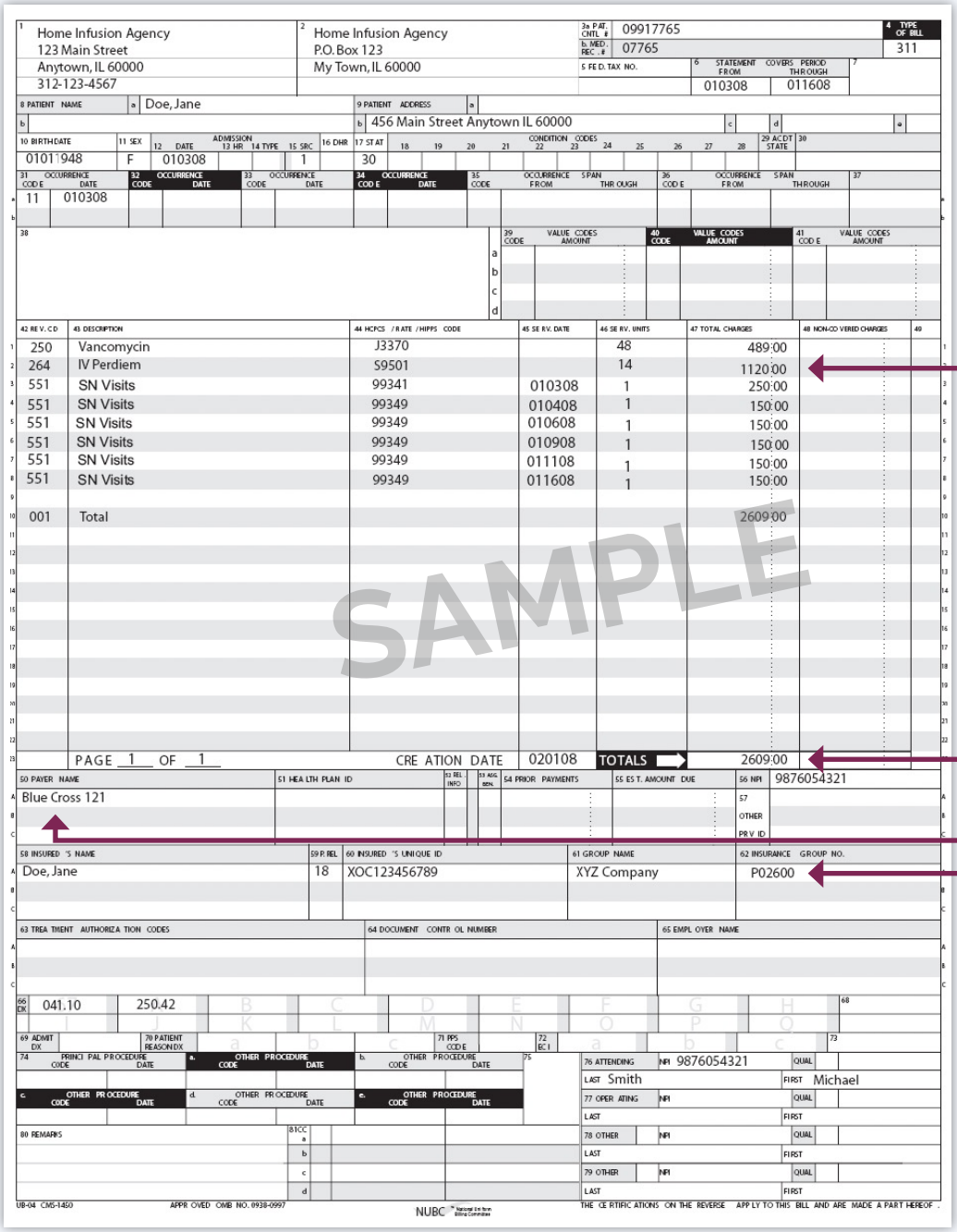
This shows the total payment made by your health insurance

The adjustment reflects the amount the hospital has agreed not to charge

This is your balance due as of the above invoice date

Use this number for questions regarding this account

# Home infusion sample bill



1 Home Infusion Agency
123 Main Street
Anytown, IL 60000
312-123-4567

2 Home Infusion Agency
P.O. Box 123
My Town, IL 60000

3a PAT. CNTL # 09917765
b. MED. REC. # 07765
4 TYPE OF BILL 311
5 FED. TAX NO.
6 STATEMENT COVERS PERIOD FROM 010308 THROUGH 011608

8 PATIENT NAME: Doe, Jane
9 PATIENT ADDRESS: 456 Main Street Anytown IL 60000
10 BIRTHDATE 0101 1948
11 SEX F
12 ADMISSION DATE 010308
13 HR
14 TYPE
15 SRC 1
16 DHR
17 STAT 30
18 19 20 21 CONDITION CODES 22 23 24 25 26 27 28
29 ACDT STATE
30
31 OCCURRENCE CODE 11 DATE 010308
32 OCCURRENCE CODE DATE
33 OCCURRENCE CODE DATE
34 OCCURRENCE CODE DATE
35 OCCURRENCE SPAN CODE FROM THROUGH
36 OCCURRENCE SPAN CODE FROM THROUGH
37
38
39 VALUE CODES CODE AMOUNT
40 VALUE CODES CODE AMOUNT
41 VALUE CODES CODE AMOUNT

| 42 REV. CD | 43 DESCRIPTION | 44 HCPCS / RATE / HIPPS CODE | 45 SERV. DATE | 46 SERV. UNITS | 47 TOTAL CHARGES | 48 NON-COVERED CHARGES |
|---|---|---|---|---|---|---|
| 250 | Vancomycin | J3370 | | 48 | 489.00 | |
| 264 | IV Perdiem | S9501 | | 14 | 1120.00 | |
| 551 | SN Visits | 99341 | 010308 | 1 | 250.00 | |
| 551 | SN Visits | 99349 | 010408 | 1 | 150.00 | |
| 551 | SN Visits | 99349 | 010608 | 1 | 150.00 | |
| 551 | SN Visits | 99349 | 010908 | 1 | 150.00 | |
| 551 | SN Visits | 99349 | 011108 | 1 | 150.00 | |
| 551 | SN Visits | 99349 | 011608 | 1 | 150.00 | |
| 001 | Total | | | | 2609.00 | |

PAGE 1 OF 1 CREATION DATE 020108 TOTALS 2609.00

SAMPLE

50 PAYER NAME: Blue Cross 121
51 HEALTH PLAN ID
52 REL INFO
53 ASG BEN
54 PRIOR PAYMENTS
55 EST. AMOUNT DUE
56 NPI 9876054321
57 OTHER PRV ID

| 58 INSURED'S NAME | 59 P REL | 60 INSURED'S UNIQUE ID | 61 GROUP NAME | 62 INSURANCE GROUP NO. |
|---|---|---|---|---|
| Doe, Jane | 18 | XOC123456789 | XYZ Company | P02600 |

63 TREATMENT AUTHORIZATION CODES
64 DOCUMENT CONTROL NUMBER
65 EMPLOYER NAME
66 DX 041.10 250.42
67 A B C D E F G H I J K L M N O P Q
68
69 ADMIT DX
70 PATIENT REASON DX
71 PPS CODE
72 ECI a b c
73
74 PRINCIPAL PROCEDURE CODE DATE
a. OTHER PROCEDURE CODE DATE
b. OTHER PROCEDURE CODE DATE
75
c. OTHER PROCEDURE CODE DATE
d. OTHER PROCEDURE CODE DATE
e. OTHER PROCEDURE CODE DATE
76 ATTENDING NPI 9876054321 QUAL LAST Smith FIRST Michael
77 OPERATING NPI QUAL LAST FIRST
78 OTHER NPI QUAL LAST FIRST
79 OTHER NPI QUAL LAST FIRST
80 REMARKS
81CC a b c d

UB-04 CMS-1450 APPROVED OMB NO. 0938-0997 NUBC THE CERTIFICATIONS ON THE REVERSE APPLY TO THIS BILL AND ARE MADE A PART HEREOF.

Services including the infusion and care before and after

Total cost for all medical services related to the infusion

Your insurance information

Your identification as it relates to your insurance, including your unique ID, group name, and insurance group number

# How plans cover specialty medications

Some drugs are known as **specialty drugs**. These medications require special care and are usually distributed through a **specialty pharmacy or specialty distributor** to ensure they are handled safely. Specialty medications may have special storage requirements or involve infusions or injections. Depending on the plan, specialty products may be covered under the medical benefit, the pharmacy benefit, or both.[26]

Both private and public insurance offer medical and pharmacy benefits. Each type of benefit covers different products and services, for example[26]:

## Medical benefit

often covers procedures or care received in a hospital or at a doctor's office.[26]

- Medications administered in an **outpatient setting** (eg, home, hospital, doctor's office, freestanding infusion clinic) may incur additional costs for the visit[26]
- **Infusions are usually covered under the medical benefit** when they are administered in a clinic or office setting by a provider.[27] Medication infused in the home may be covered under either the medical benefit or the pharmacy benefit, depending on your health plan's benefit design[27]


## Pharmacy benefit

often covers medications picked up from a retail or mail-order pharmacy.[26]

- Under the pharmacy benefit, you may hear the term ***limited distribution***. This means that there may only be a few specific pharmacies that will ship the medication to the patient's home. This is most common with novel therapies that have complex shipping and handling requirements[27]

# Specialty pharmacy

A **specialty pharmacy** is a type of pharmacy that provides services and dispenses medications for complex health conditions like Duchenne that require specialty medications like injections or infusions. For Duchenne therapy, the role of the specialty pharmacy is to:

- **Work with the doctor's office** and your insurance company to distribute the specialty medication.
- **Provide counseling** on treatment, including managing side effects.
- **Help with financial** assistance options.
- **Assist with setting up** home infusions and dispensing drugs and supplies.

Before shipping the medication, the specialty pharmacy will obtain **prior authorization** from the insurer to confirm the medication is covered and being administered according to the insurance company's policies. This can sometimes delay the start of therapy.

**Your SareptAssist Case Manager can help you determine how your therapy is covered and help you navigate the prior authorization process.**

## Outpatient vs home infusion

Delivery of Duchenne medication varies by plan, coverage type, location of treatment, and your doctor's prescribing orders. In general, there are a few terms that are helpful to know:

| OUTPATIENT | HOME INFUSION |
|---|---|
| **BUY AND BILL**<br>The medication is purchased by the provider from a specialty distributor and administered in an office or outpatient setting. In this case, you pay the co-pay and deductible, if applicable, to the doctor's office for treatment and administration.[28] | Patients receiving their medication at home will pay their doctor's office the co-pay, deductible, and co-insurance, if applicable. The patient's medication will be shipped to their home for administration.[28]<br>The specialty pharmacy is responsible for delivering the medication and setting up the home health nursing.[29] |

**Your SareptAssist Case Manager can help you navigate your drug delivery process, coordinate home health and nursing visits, and provide financial assistance options depending on eligibility.**

# Drug formularies, prior authorization, and reauthorization

## What is a formulary?

Most health plans have a **formulary as part of their pharmacy benefit**. The formulary is a list of prescription drugs that is grouped into different categories or tiers. The amount you may have to pay for the drug is determined by the tier level. Typically, a drug formulary includes **4 or 5 tiers**. Here's an example of a typical tier structure[30]:

| | |
|---|---|
| **TIER 1** | **Generic or lowest cost drugs, including some corticosteroids** |
| **TIERS 2-3** | **Brand-name drugs or more expensive generics** |
| **TIER 4** | **More expensive brand-name drugs** |
| **TIER 5** | **Specialty drugs such as infusions or the most expensive medications**[31]<br>Some specialty drugs administered by a provider or in the outpatient setting are covered by the **medical benefit**.[26] Out-of-pocket costs may differ depending on whether a drug is covered under the pharmacy or medical benefit. |

## What is a prior authorization?

Most formularies include restrictions such as **prior authorizations**, which are processes that health plans use to ensure the drug is being used appropriately and to better control the use of more expensive medications.[32]

If your health plan requires a prior authorization, it means that your doctor must submit documentation explaining why the prescribed Duchenne therapy is necessary before the plan will cover it. **Prior authorization requirements vary by plan.**

## What is reauthorization?

Your health plan will approve exon-skipping therapy for a limited period of time, typically 6 months to 1 year. After that period, reauthorization will be required for the patient to continue therapy. It will be based on results from potential lab work and clinical assessments.[33] Your doctor will schedule regular assessments to gather the clinical information your insurance plan needs for reauthorization. **Missed appointments can impact the reauthorization process and interrupt your treatment schedule.**

**Your doctor, their office staff, and your SareptAssist Case Manager can help guide you through the prior authorization and reauthorization process. The next section provides more details on this topic.**

# The journey to accessing Duchenne treatment

Once you and your doctor decide that treatment is right for you or your family member, Sarepta has resources that can help you navigate the complexities of accessing treatment. The process is different for everyone. It varies due to many factors, such as the type of insurance you have, your treatment needs, and financial assistance options. It will be important to get to know your healthcare team, who will be your partners in your treatment journey and can help you with your specific situation.

## Who is on your care team?

**At the doctor or clinic**

**Doctor:** prescribes medication and provides medical care.

**Nurse:** provides care, administers medication.

**Office Staff:** handles appointments and administrative issues such as billing and payments; assists with prior authorizations and appeals to help patients get access to medications.

**At the health plan**

**Member Services:** handles insurance policy questions and claims processing.

**Medical Director:** responsible for helping to build formularies and reviewing prior authorizations and appeals.

**Your Sarepta team**

**SareptAssist Case Manager (CM):** one of your key contacts for questions about your insurance benefits, treatment delivery, and access to medication.

**Patient Access Manager (PAM):** works with you and your SareptAssist Case Manager to navigate the denial and appeals process.

**Director Market Access and Reimbursement (DMAR):** provides access and reimbursement support for doctors' offices and insurance companies.

**Duchenne Nurse Educator (DNE):** provides training for drug administration.

**Specialty pharmacy/ drug distributor**

**Pharmacist:** coordinates medication shipment, reviews physician orders, and consults with families if needed.

**Nurse/Coordinator:** facilitates contracts/appointments with home health agencies.

**Dedicated Care Team:** coordinates medication shipment to site or home, ships necessary supplies, and assists with home healthcare coordination.

# Important steps in accessing exon-skipping therapy

Once you and your doctor have decided that exon-skipping therapies are the best option for you, there are **4 key steps to gain access** to treatment. Your SareptAssist Case Manager will be with you every step of the way.

STEP 1

## Submitting an enrollment form

This is the process to determine the type of insurance you have and the expected coverage it provides for the prescribed Duchenne therapy, including prior authorization policies and out-of-pocket expenses. Your SareptAssist Case Manager will help you understand your specific insurance benefits and next steps. **Refer to the next few pages for an overview of the authorization process for Duchenne medications.**

STEP 2

## Treatment location options

Patients may receive infusions at an infusion center or at home. You and your doctors should discuss these options, including whether home therapy is an option for your family. Some insurance plans may only cover outpatient infusions or only cover home infusions.

STEP 3

## Starting treatment

Once your insurance benefits have been confirmed and approval for infusions has been obtained, your SareptAssist Case Manager will work closely with you to facilitate treatment access and coordinate drug delivery to your treatment location. This process will vary according to the type of pharmacy and location of the infusion. Then, your Duchenne Nurse Educator will guide you and your healthcare team through the drug administration process.

**Remember that it is important to do your part by showing up for appointments, taking your medications on time, and completing assessments to avoid delays in treatment.**

STEP 4

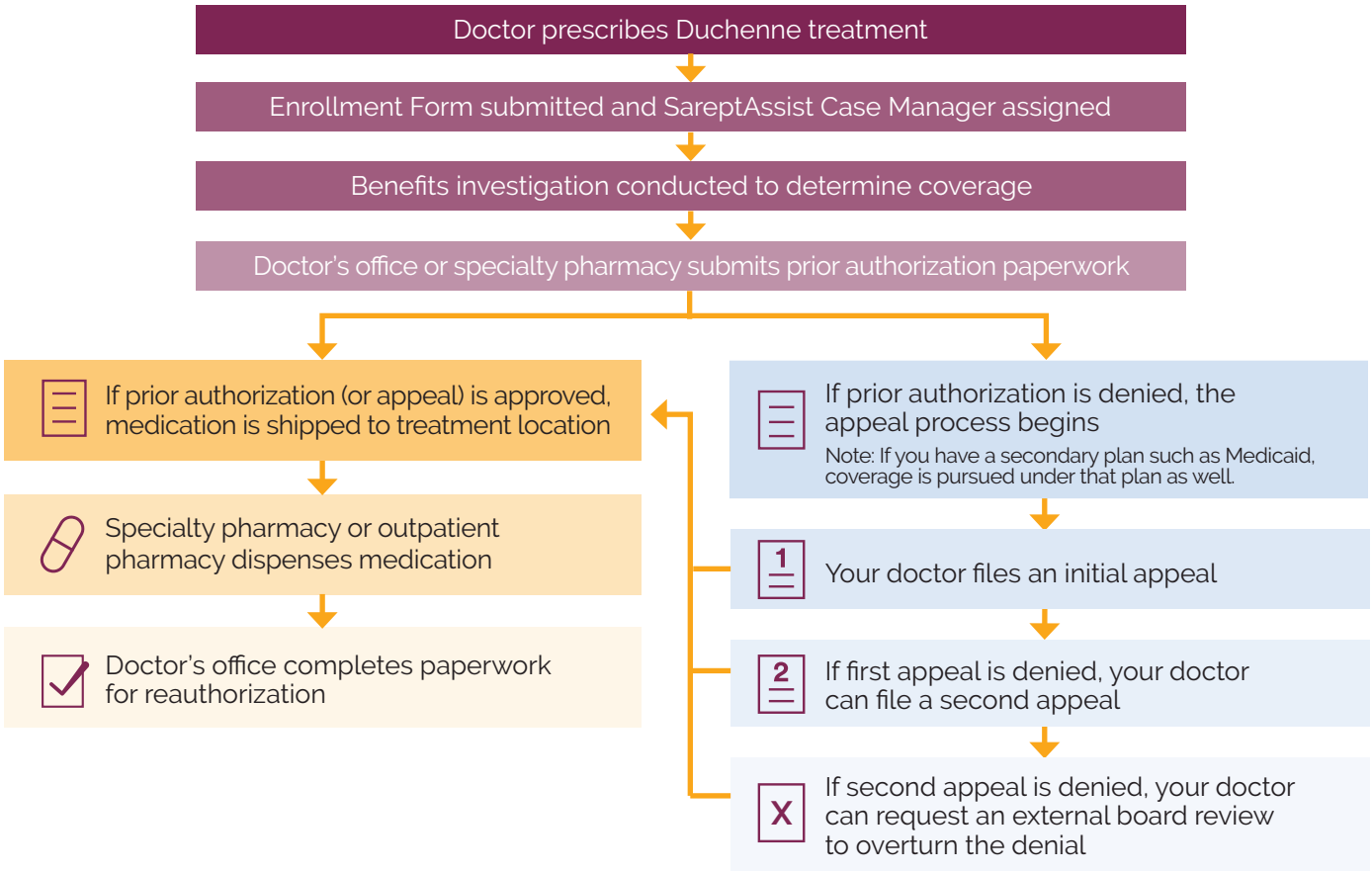
## Ongoing support

Your SareptAssist Case Manager is committed to working with you during your treatment journey, and will check in with you periodically. As your needs change (eg, you have new insurance, a change of address, are planning a vacation), your SareptAssist Case Manager can keep you informed of your options to help avoid treatment interruptions.

**Always consult with your physician before making any decisions about your Duchenne treatment regimen.**

# Benefits investigation: Duchenne medication authorization process

The first major step in accessing treatment is obtaining a prior authorization from your insurance company. This can be a long and difficult process, but your SareptAssist Case Manager will be there to help you from the beginning. To better understand what typically happens during the authorization process, a brief overview has been provided below.




## What can you do?

**You are a critical part of your own healthcare team. Actions you can take while going through the benefits investigation process include:**

- Calling your SareptAssist Case Manager at 1-888-SAREPTA (1-888-727-3782) Monday through Friday, 8:30 AM - 6:30 PM ET
- Contacting your insurance company directly with questions about coverage
- Sharing any communication you may receive from your insurer with your SareptAssist Case Manager and doctor's office
- Checking in regularly with your doctor's office or office staff at your infusion center
- Following up with scheduled clinical appointments with your doctor's office for necessary evaluations
- Reaching out to patient advocacy organizations for assistance
- Sharing your experiences

# Common reasons for denials

## Why is a prior authorization denied?

**There are a number of reasons why prior authorization may be denied for Duchenne treatment. Common ones include:**

- Medication is only covered for a specific age range
- Incomplete information was provided, such as lack of proper clinical assessments
- The patient has not been on a treatment regimen of steroids
- Drug is excluded from coverage
- Duchenne therapy is not considered medically necessary based on the plan's specific criteria. For example[34]:
  - Genetic testing is required to confirm a gene mutation for Duchenne
  - There are specific requirements for respiratory function such as the ability to breathe without the use of a ventilator
  - A specific score range is required on functional tests as defined by the insurance company
  - The plan may not approve a newly FDA-approved medication

## Why is a reauthorization denied?

**Common reasons include:**

- Patient did not go to appointments or complete the necessary clinical assessments as required by the insurance company to assess therapeutic outcomes
- Specific assessments (eg, score on 6MWT) were not met
- Paperwork mistake or missing information

6MWT=6-minute walk test.

# Financial assistance options

Depending on the type of insurance you have and other relevant circumstances, you may qualify for financial assistance programs. Your SareptAssist Case Manager can help provide information about the options and see if you are eligible for the following Sarepta programs.


## Patient co-pay assistance program

This program may help with some out-of-pocket costs for Duchenne medication (not the costs of supplies or other treatment-related costs). It is for eligible individuals in the United States with commercial insurance only, not government insurance, such as Medicaid, who are prescribed treatment with a Sarepta product.

**Speak with your SareptAssist Case Manager about which financial assistance options may be best for you based on your needs.**

## Multiple independent charitable organizations

There are multiple programs that offer financial assistance, such as the Duchenne Family Assistance Program.

**Your SareptAssist Case Manager can provide more information and direct you to these third-party options.**

**You can rely on your SareptAssist Case Manager and care team to stay informed of your options and help avoid treatment interruptions as your needs change (eg, new insurance, change of address).**

# Real cases

## Different patients, different journeys to Duchenne therapy

While you are not alone in your diagnosis, every path to therapy is unique. That's why it's so important to speak with a SareptAssist Case Manager as soon as you are prescribed your medication. Your personalized SareptAssist Case Manager has a team backed with years of knowledge and experience managing many different case dynamics and circumstances.

Following are examples of the treatment journey for our patients, including the challenges in gaining approval, and how the Sarepta team can help—so you can see the different ways the path to access can unfold, and what you may need to consider along the way.

**Please note that these examples are for recurring therapies only.**

**Darren**
**Living with Duchenne**

# Case study #1

**Patient:** Boy, age 5 **Type of insurance:** Medicaid fee-for-service

| | Access journey | Role of care team |
|---|---|---|
| **Month 1** | • Physician completed and submitted the SareptAssist Enrollment Form<br>• Prior authorization was submitted by the doctor's office for the patient, along with the genetic testing report and clinical notes from his most recent visit with his MD—including functional assessments that the plan requires for approval<br>• The health plan then approved his therapy, requiring reauthorization every 6 months | • CM completed welcome call with the parents and re-confirmed this patient would be getting infused at an infusion clinic near his home<br>• CM informed parents that insurance approval may take several weeks, and kept them informed throughout the process<br>• CM obtained insurance benefits through Medicaid<br>• DMAR reviewed the policy and prior authorization criteria with the office and worked with the site of care to determine procurement preference and infusion plans<br>• The infusion center preferred to white bag, so the CM helped coordinate necessary information to the SP<br>• As the patient has not received this medication previously, the CM notified the DNE to provide further education on DMD and the infusion process to the patient and his parents |
| **Month 2** | • Patient began infusions in the outpatient infusion clinic per the decision between the family and their physician | • CM confirmed with infusion site and family that everything was ready to begin infusion<br>• DNE provided infusion education for infusion nurse at the outpatient clinic prior to first infusion |
| **Month 8** | • Reauthorization was submitted and approved every 6 months | • DMAR educated office staff on reauthorization procedures and proper documentation<br>• CM advised the patient to make an appointment with their doctor to obtain the necessary functional assessments and updated chart notes necessary for reauthorization |

CM=case manager; DMAR=Director Market Access and Reimbursement; DMD=Duchenne muscular dystrophy; DNE=Duchenne nurse educator; MD=doctor of medicine; SP=specialty pharmacy.

# Case study #2

**Patient:** Spanish-speaking male, age 31 **Type of insurance:** Medicaid fee-for-service

| | Access journey | Role of care team |
|---|---|---|
| **Month 1** | • Physician completed and submitted the SareptAssist Enrollment Form<br>• Prior authorization was submitted by the doctor's office with the genetic testing report for the patient<br>• Duchenne therapy was denied by the health plan due to his age and because he required further lab work documenting renal function within the last 3 months<br>• The first appeal was submitted by office staff, which included updated lab work and a letter of medical necessity from the doctor's office | • SareptAssist CM obtained insurance benefits through Medicaid and connected with family to discuss next steps<br>• DMAR reviewed policy and prior authorization criteria with the office<br>• CM sent referral to the SP to start the authorization process with the doctor's office<br>• CM included the PAM once initial authorization was denied. A Spanish-speaking PAM spoke with the patient to discuss writing a patient appeal letter to be included with the doctor's letter<br>• PAM provided the patient with guidance, explaining how it would be necessary for the patient to obtain the required lab work to document renal function and where he could go to complete that<br>• DMAR worked with the office to help them understand the denial and educated them on the appeal process and documentation required to support the case for coverage |
| **Month 2** | • The health plan approved therapy for in-home use per the patient's request, which required reauthorization every 3 months | • DNE performed in-service for the home health nurse prior to first infusion, and then followed up after infusion to ask how it went<br>• CM worked with SP and family to prepare for first infusion including home health nursing confirmation, appointment setup, and ensuring drugs and supplies were at the patient's home |
| **Month 6** | • Reauthorization submitted by the doctor's office with a letter of medical necessity and updated clinical notes<br>• There was no interruption to therapy and a subsequent reauthorization was approved for 1 year | • DMAR educated office staff on reauthorization procedures and proper documentation<br>• CM continued to check in with family and to monitor upcoming reauthorizations |

CM=case manager; DMAR=Director Market Access and Reimbursement; DNE=Duchenne nurse educator; PAM=patient access manager; SP=specialty pharmacy.

# Case study #3

**Patient:** Boy, age 12 **Type of insurance:** Managed Medicaid

| | Access journey | Role of care team |
|---|---|---|
| **Month 1** | • Physician completed and submitted the SareptAssist Enrollment Form<br>• Prior authorization was submitted by the doctor's office with the genetic testing report for the patient, but Duchenne therapy was denied by the health plan due to "missing clinical support (6-minute walk test [6MWT])"<br>• Office staff then submitted the first appeal | • DMAR reviewed the policy and prior authorization criteria with the MDO<br>• CM obtained consent connected with family to discuss the process<br>• Insurance benefits were obtained and information was provided to the doctor's office to assist with the initial authorization submission and the first appeal<br>• DMAR provided education to MDO on how to process prior authorization appeals and aligned upon next steps |
| **Month 2** | • The first appeal was denied because 6MWT was not included with the appeal<br>• The patient was unable to complete the 6MWT by the time of submission so a second appeal was submitted, heading straight to an external review (some Medicaid plans only offer one internal appeal) | • CM brought in PAM to work with family on the appeal process<br>• PAM met in person with the patient's mother to educate on documentation needs and about the external review process<br>• DMAR educated the provider on the external review process and timelines as well as necessary documentation<br>• Once confirmation of external review request was submitted by medical doctor's office, CM/PAM followed up on status |
| **Month 7** | • The external review board overturned the decision, allowing the patient to receive coverage for therapy and requiring reauthorization every 6 months | • DNE performed in-service for the clinic prior to first infusion, and then followed up after infusion to ask how it went. DNE then worked with an SP to identify a home health nurse<br>• CM confirmed the infusion site received the drug shipment and family had scheduled appointments |
| **Month 8** | • Multiple reauthorization requests have been processed, with occasional denials due to ambulation status; however, each denial was overturned after the submission of an appeal | • CM continued to check in with family and monitor reauthorizations<br>• DMAR confirmed next appointment visit for CM and educated office on requirements for appeals<br>• CM coordinated support for family as needed |

CM=case manager; DMAR=Director Market Access and Reimbursement; DNE=Duchenne nurse educator; MDO=medical doctor's office; PAM=patient access manager; SP=specialty pharmacy.

# Case study #4

**Patient:** Boy, age 9 **Type of insurance:** Commercial

| | Access journey | Role of care team |
|---|---|---|
| **Month 1** | • Physician completed and submitted the SareptAssist Enrollment Form<br>• Prior authorization was submitted by the doctor's office with the genetic testing report for the patient, but Duchenne therapy was denied because his healthcare policy listed it as "not medically necessary" | • DMAR reviewed the policy and prior authorization criteria with the MDO<br>• CM obtained benefits and completed welcome call with family to discuss next steps and confirmed plan to start therapy at hospital<br>• CM sent referral to SP to start authorization process with MDO after DMAR confirmed the site preference to accept drug from SP |
| **Month 3** | • A letter of medical necessity appeal was submitted to the insurance company<br>• The appeal was denied again, citing the medical policy that stated "medication was not medically necessary"<br>• A second appeal was submitted, which headed straight to an external review | • DMAR educated office on appeal process and requirements based on policy<br>• After denial, CM looped in PAM to educate family on the appeals process and steps they could take<br>• PAM educated family on steps they could take with MD appeal<br>• DMAR educated the provider on the external review process and timelines as well as necessary documentation |
| **Month 6** | • The external review board overturned the previous decision, allowing the patient to receive coverage for therapy and requiring authorization every 6 months | • CM helped family secure an infusion location and maintained regular check-ins to ensure information was up to date for reauthorization |
| **Month 11** | • The first reauthorization was submitted and denied, claiming the patient did not meet the plan's improvement criteria | • DMAR confirmed next appointment visit for CM and educated office on requirements for the appeal |
| **Month 13 and beyond** | • After a doctor's appointment, which allowed for the submission of recent clinical notes, the reauthorization was appealed<br>• The health plan approved Duchenne therapy for 1 year, with reauthorization required after 1 year | • CM continued to check in with family and educate/monitor on upcoming reauthorizations |

CM=case manager; DMAR=Director Market Access and Reimbursement; MD=doctor of medicine; MDO=medical doctor's office; PAM=patient access manager; SP=specialty pharmacy.

SAREPTASSIST

# How Sarepta can help

SareptAssist is a support program designed to help patients seeking information about exon-skipping therapies.

## Our dedicated team will provide information on:

- Insurance benefits
- Financial assistance options
- Treatment logistics
- Options for weekly infusions
- Ongoing education and support

Remember, while your doctor is your first point of contact for all medical needs, your **SareptAssist Case Manager** is committed to working with you during the treatment journey and will check in regularly. **For more information, visit SareptAssist.com.**

**We are committed to partnering with you, your doctors, and your health plan to ease the stress of accessing exon-skipping therapy for Duchenne, so you can focus on what's important—life with your family.**


**References: 1.** Popplewell LJ, Trollet C, Dickson G, Graham IR. Design of phosphorodiamidate morpholino oligomers (PMOs) for the induction of exon skipping of the human *DMD* gene. *Mol Ther.* 2009;17(3):554-561. **2.** What is health insurance? Healthinsurance.org. Accessed January 19, 2024. https://www.healthinsurance.org/glossary/health-insurance/ **3.** What is self funding? Health Care Administrators Association. Accessed January 19, 2024. https://www.hcaa.org/page/selffunding **4.** Medicaid eligibility. Medicaid.gov. Accessed February 1, 2024. https://www.medicaid.gov/medicaid/eligibility/index.html **5.** People dually eligible for Medicare and Medicaid. Centers for Medicare & Medicaid Services. March 2023. Accessed February 1, 2024. https://www.cms.gov/medicare-medicaid-coordination/medicare-and-medicaid-coordination/medicare-medicaid-coordination-office/downloads/mmco_factsheet.pdf **6.** Worstell C. How Medicaid works as secondary insurance. Medicare Advantage. January 12, 2024. Accessed March 7, 2024. https://www.medicareadvantage.com/medicaid/medicaid-as-secondary-insurance **7.** Poverty guidelines. Office of the Assistant Secretary for Planning and Evaluation. Accessed March 7, 2024. https://aspe.hhs.gov/topics/poverty-economic-mobility/poverty-guidelines **8.** Medicaid. Medicaid.gov. Accessed January 19, 2024. https://www.medicaid.gov/medicaid/index.html **9.** Provider payment and delivery systems. MACPAC. Accessed January 19, 2024. https://www.macpac.gov/medicaid-101/provider-payment-and-delivery-systems/ **10.** Medicare program - general information. Centers for Medicare & Medicaid Services. Accessed January 19, 2024. https://www.cms.gov/Medicare/Medicare-General-Information/MedicareGenInfo **11.** Get started with Medicare. Medicare.gov. Accessed January 19, 2024. https://www.medicare.gov/basics/get-started-with-medicare **12.** Parts of Medicare. Medicare.gov. Accessed February 1, 2024. https://www.medicare.gov/basics/get-started-with-medicare/medicare-basics/parts-of-medicare **13.** Medicare eligibility for disabled youths. Medicare Interactive. Accessed January 19, 2024. https://www.medicareinteractive.org/get-answers/medicare-basics/medicare-eligibility-overview/medicare-eligibility-for-disabled-youths **14.** Benefits for children with Duchenne muscular dystrophy. Harold W. Conick & Associates Ltd. Accessed February 1, 2024. https://conicklaw.com/benefits-for-children-with-duchenne-muscular-dystrophy/ **15.** Advising Congress on Medicaid and CHIP Policy: State Children's Health Insurance Program (CHIP). February 2018. Accessed January 19, 2024. https://www.macpac.gov/wp-content/uploads/2018/02/State-Children%E2%80%99s-Health-Insurance-Program-CHIP.pdf **16.** Compassionate allowances. Social Security Administration. Accessed February 5, 2024. https://www.ssa.gov/compassionateallowances/ **17.** Figueiredo M. DMD adults added to SSA compassionate allowance program. Muscular Dystrophy News Today. August 19, 2021. Accessed January 19, 2024. https://musculardystrophynews.com/2021/08/19/social-security-compassionate-allowances-program-eligibility-duchenne-muscular-dystrophy/ **18.** Duchenne muscular dystrophy - adult. Disability Benefits Center. Accessed January 19, 2024. https://www.disabilitybenefitscenter.org/compassionate-allowances/duchenne-muscular-dystrophy-adult **19.** Musumeci M, Watts MO, Ammula M, Burns A. Medicaid financial eligibility in pathways based on old age or disability in 2022: findings from a 50-state survey. KFF. July 11, 2022. Accessed February 1, 2024. https://www.kff.org/report-section/medicaid-financial-eligibility-in-pathways-based-on-old-age-or-disability-in-2022-findings-from-a-50-state-survey-appendix/ **20.** Shapiro J. Katie Beckett defied the odds, helped other disabled kids live longer. NPR. May 21, 2012. Accessed January 19, 2024. https://www.npr.org/sections/healthshots/2012/05/21/153202340/katie-beckett-defied-theodds-helped-other-disabled-kids-live-longer **21.** Health insurance basics. Centers for Medicare & Medicaid Services. Accessed February 1, 2024. https://www.cms.gov/files/document/nsa-health-insurance-basics.pdf **22.** Deductible. HealthCare.gov. Accessed January 19, 2024. http://www.healthcare.gov/glossary/deductible/ **23.** What is coinsurance? MetLife. August 22, 2023. Accessed February 1, 2024. https://www.metlife.com/stories/benefits/what-is-coinsurance/ **24.** Deductible vs. copayment: what's the difference? Verywell Health. Updated July 21, 2023. Accessed January 19, 2024. https://www.verywellhealth.com/deductible-vs-copayment-whats-the-difference-1738550 **25.** Cost sharing. Cigna. Accessed January 19, 2024. https://www.cigna.com/employers/insights/informed-on-reform/cost-sharing **26.** McCain J. The importance of a thorough benefits investigation to help navigate medical vs pharmacy benefit. National Association of Medication Access & Patient Advocacy, Inc. Accessed January 19, 2024. https://namapa.org/medical-vs-pharmacy-benefit **27.** American Society of Health-System Pharmacists. ASHP Specialty Pharmacy Resource Guide. December 2015. Accessed January 19, 2024. https://www.ashp.org/-/media/assets/pharmacy-practice/resource-centers/specialty-pharmacy/specialty-pharmacy-resource-guide.ash **28.** Specialty drug acquisition models. Specialty Drug Resource. Accessed January 19, 2024. https://www.specialtydrugresource.com/coverage-landscape-overview/buy-and-bill-vs-specialty-pharmacy **29.** About home and alternate site infusion. National Home Infusion Association. Accessed February 1, 2024. https://nhia.org/about-infusion-therapy/ **30.** Torrey T. What is a health insurer's drug formulary and tier pricing? Verywell Health. Updated February 26, 2020. Accessed February 1, 2024. https://www.verywellhealth.com/drug-formulary-tiers-pricing-health-insurance-plans-2615042 **31.** What is a prescription drug tier list (formulary) for insurance? MetLife. September 20, 2023. Accessed February 1, 2024. https://www.metlife.com/stories/benefits/drug-tier-list/ **32.** Bihari M. Prior authorization: overview, purpose, process. Verywell Health. Updated July 27, 2023. Accessed February 1, 2024. https://www.verywellhealth.com/prior-authorization-1738770 **33.** CVS Caremark. Specialty guideline management: exondys 51 (eteplirsen). Accessed January 19, 2024. https://www.hopkinsmedicine.org/-/media/johns-hopkins-health-plans/documents/ppmco/pp_exondys_criteria.pdf **34.** Margaretos NM, Bawa K, Engmann NJ, Chambers JD. Patients' access to rare neuromuscular disease therapies varies across US private insurers. *Orphanet J Rare Dis.* 2022;17(1):36.


SAREPTA THERAPEUTICS