



NORD®
National Organization
for Rare Disorders

NATIONAL POLICY & ADVOCACY TASKFORCE MEETING

September 12, 2023

National Organization for Rare Disorders | rarediseases.org

The National Organization for Rare Disorders (NORD) is the leading independent patient advocacy organization representing all individuals and families affected by rare diseases in the United States.



MEETING AGENDA

- Housekeeping Items
- Individual Care Challenges & NORD Information Services
- Introduction to new members of NORD's Policy Team
- Policy Updates & Year-to-Date Highlights
- Implementation of the Inflation Reduction Act
- Medicaid Unwinding

2023 TASKFORCE MEETING SCHEDULE



REGIONAL MEETINGS

- Week of November 6 or November 13
- Week of December 11

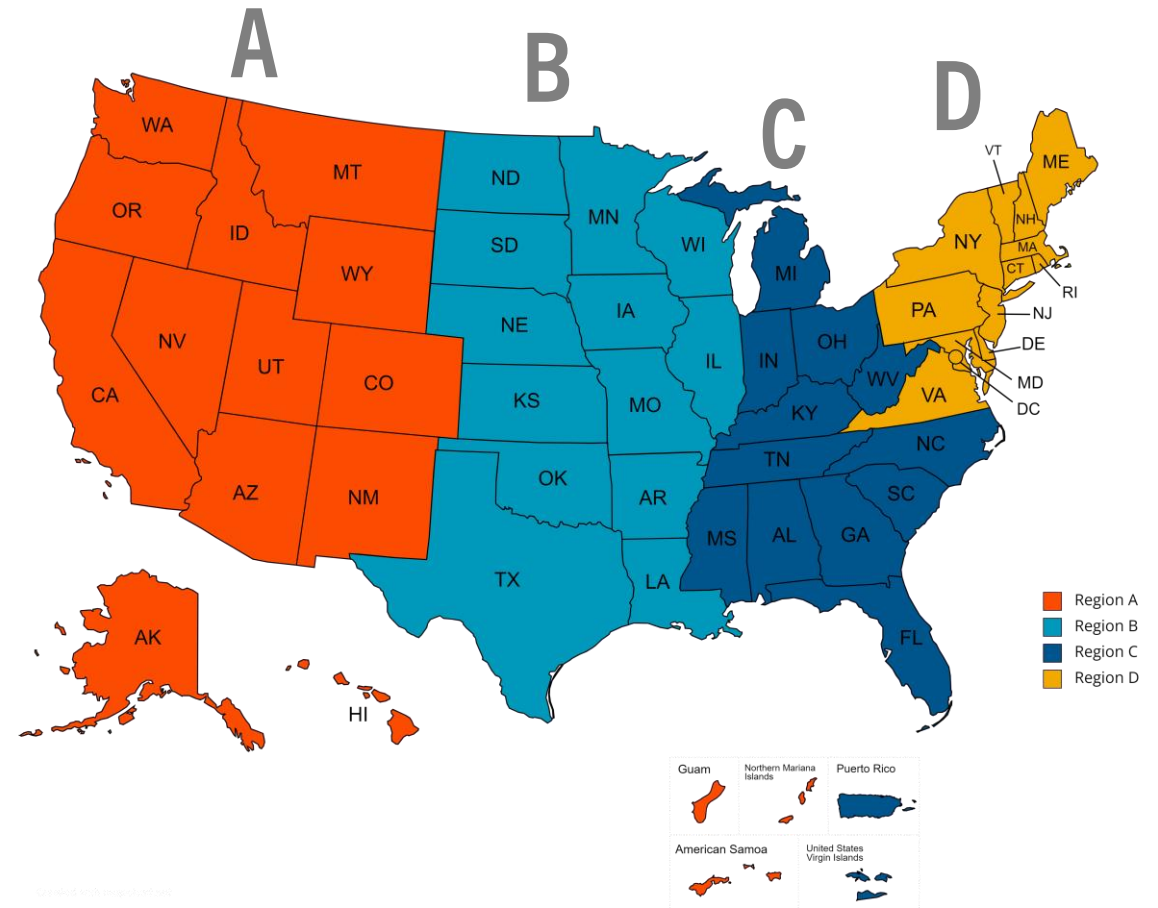
NATIONAL MEETINGS

- September 12
- Early 2024

UPCOMING REGIONAL MEETINGS

OCTOBER REGIONAL MEETINGS WILL BE RESCHEDULED TO NOVEMBER

- Meetings will focus on a year-end recap and looking ahead to next year
- Keep an eye out for an email containing specific dates and times for November Regional meetings



WHAT ARE INDIVIDUAL CARE CHALLENGES?

- Examples of individual care challenges include challenges with insurance benefits, care navigation/case management, Social Security, etc.
- While the policy work we do at NORD aims to address care challenges, NORD's policy team is not equipped to assist with individual care management
- Your elected officials can sometimes help with individual care challenges related to a state or federal agency
 - US Senate and House of Representatives offices have case work staff who may be able to help
 - If you live in the District of Columbia or a US Territory, you can contact your Congressional Representative for these services
 - Your State elected officials/staff may also be able to help with things like Medicaid

HOW CAN NORD HELP?

- NORD has several disease-specific patient & caregiver assistance programs, as well as a few disease-agnostic patient & caregiver assistance programs (ex: hurricane and natural disaster relief)
 - You can view patient assistance programs at rarediseases.org/patient-assistance-programs
- NORD's Policy & Community Engagement teams may be able to point you to resources that provide individual advocacy services. You can email us at taskforce@rarediseases.org
- NORD has a dedicated Information and Resource Services team that can be reached at 800-469-0283 or via email at informationservices@rarediseases.org

Please note that NORD provides this information for the benefit of the rare disease community. NORD is not a medical provider or health care facility, and this can neither diagnose any disease or disorder does not endorse or recommend any specific medical treatments. Patients must rely on the personal and individualized medical advice of their qualified healthcare professionals before seeking any information related to their particular diagnosis, cure or treatment of a condition or disorder.

MEET THE NORD POLICY TEAM



Heidi Ross, MPH
Vice President, Policy &
Regulatory Affairs



Karin Hoelzer, DVM, PhD
Director, Policy &
Regulatory Affairs



Allison Herrity, MPH
Policy Analyst



Hayley Mason, MPA
Policy Analyst



Mason Barrett
Policy Analyst



Supraja Sowmiyanarayanan
Program Assistant

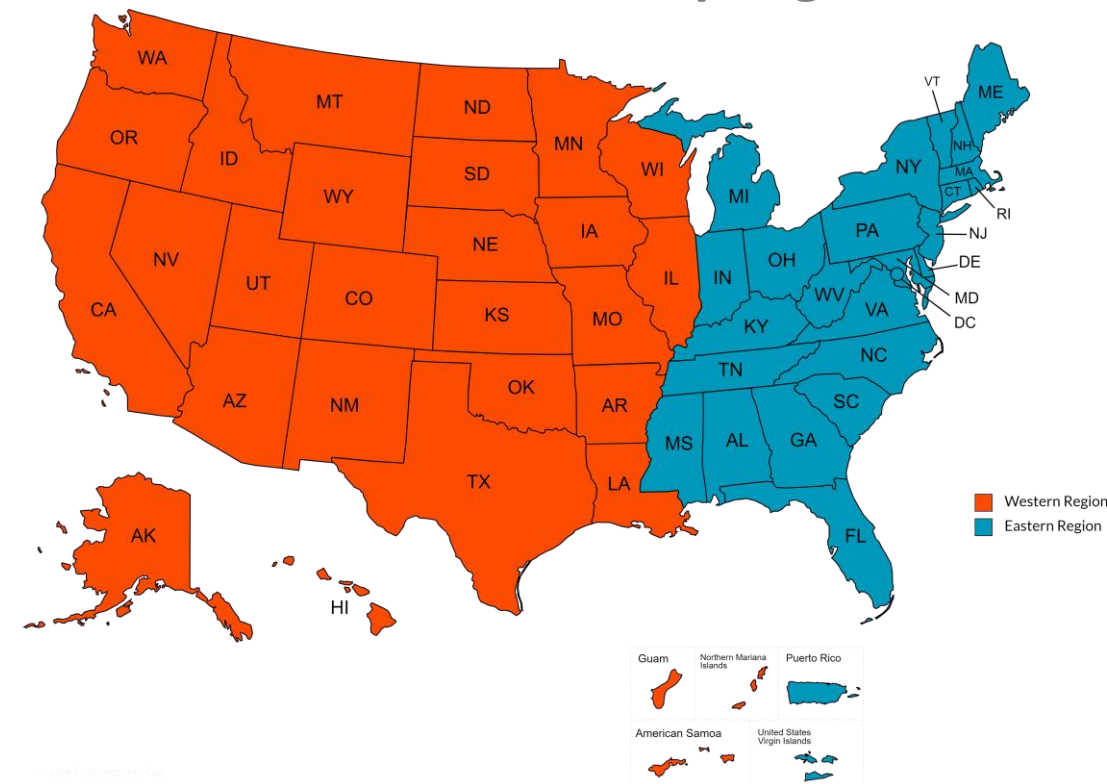


Lindsey Viscarra
State Policy Manager,
Western Region



Carolyn Sheridan, MPH
State Policy Manager,
Eastern Region

NORD State Policy Regions



RARE DISEASE POLICY UPDATES

TELEHEALTH

- Hawaii & Missouri Interstate Medical Licensure Compact legislation signed into law
- Looking to expand our telehealth work during 2024 state sessions
- Opposition to the Telehealth Benefit Expansion for Workers Act (H.R. 824)



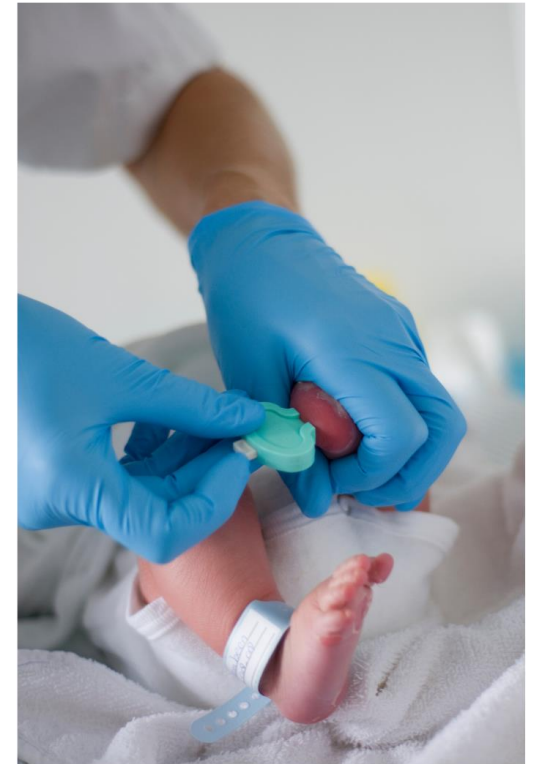
STEP THERAPY

- House and Senate bill numbers for the Safe Step Act are H.R. 2630 and S.652
- Language from the Safe Step Act was included in legislation that advanced out of the Senate HELP Committee in May
- Continue to contact your legislators!



NEWBORN SCREENING

- Working with NBS Braintrust on strategy around federal newborn screening legislation
- Currently monitoring legislation at the state level
 - Law enforcement access to NBS dried bloodspots
 - Consent for use of dried bloodspots for research purposes
- Evaluating our current position on written and informed consent for DBS retention and use
- Stay tuned for additional Newborn Screening engagement opportunities



RARE DISEASE ADVISORY COUNCILS

This legislative year **THREE** RDACs were signed into law; Indiana, Maryland, and Delaware!

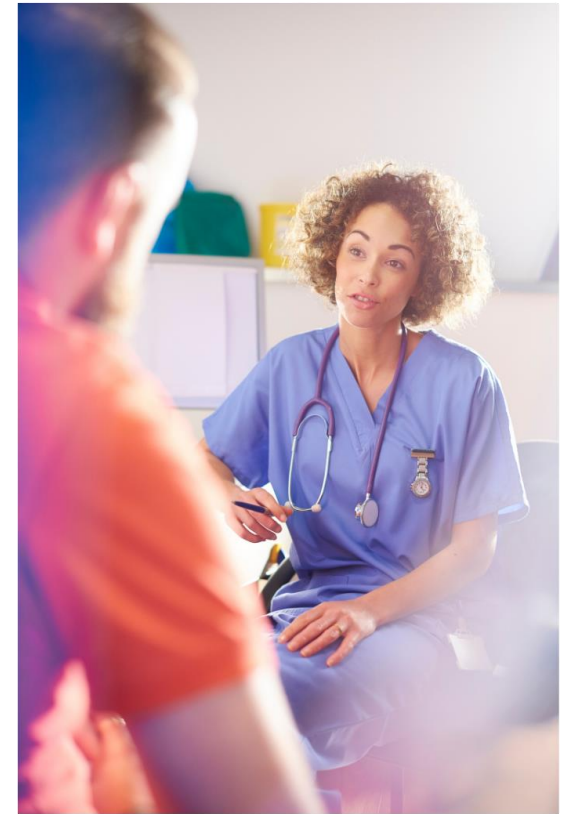
In 2024, we are kicking it into high gear! If you are in a state without an RDAC and would like to be involved in planning efforts, please email Carolyn and Lindsey at RDAC@rarediseases.org to join your state's coalition. **We can't do it without you!**



NORD Staff and Advocates were honored to join Delaware Governor John Carney in Wilmington, DE on July 26, 2023 to see the RDAC signed into law!

HEALTH COVERAGE

- NORD continues to support policies that expand access to and strengthen state Medicaid programs & oppose those that seek to restrict eligibility or benefits (ex: work requirements)
- Joined other patient organizations in urging the Administration to protect patient's access to health coverage as the Public Health Emergency ends
- Submitted comments to CMS regarding the Medicare Drug Price Negotiation Program
- Supporting biomarker coverage legislation in several states



FOOD AND DRUG ADMINISTRATION

- The Retaining Access and Restoring Exclusivity (RARE) Act passed through the Senate HELP Committee on May 11
 - This legislation clarifies the scope of the market exclusivity period for orphan drugs
- Have submitted comments on FDA regulatory issues including:
 - The design and conduct of externally controlled trials for drugs and biologics
 - Gaps in educational materials on rare disease drug development
 - Other issues such as cell and gene therapy
- Joined other patient organizations in Amicus Brief supporting FDA's authority to oversee the safety and effectiveness of drugs (Texas mifepristone case)

MEDICAID UNWINDING UPDATE

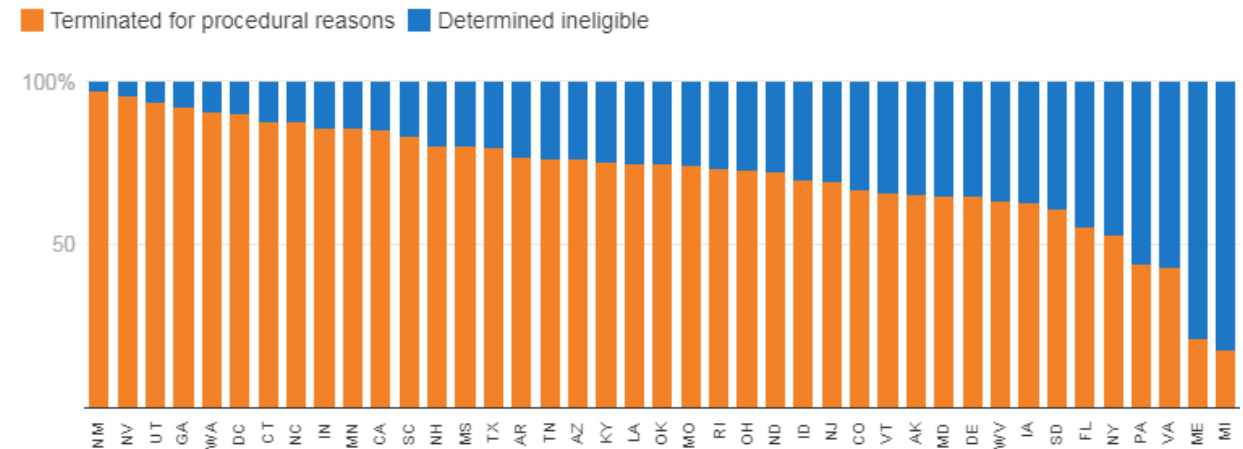
NORD is concerned about high rates of procedural disenrollments

- Most beneficiaries who have lost Medicaid coverage thus far have lost coverage due to **“procedural disenrollments.”**
- Procedural disenrollments occur when the beneficiary did not return their enrollment packets in time or did not return the packets with the requested information completed.
- High rates of procedural disenrollments are particularly concerning because many beneficiaries may not know they have lost coverage until they try and seek medical care

Figure 2

Overall, 74% of disenrollments are due to procedural reasons, among states reporting as of August 29, 2023

Of Total Disenrollments, the Share Disenrolled for Procedural Reasons vs. the Share Determined Ineligible:



NOTE: Procedural disenrollments occur when the state cannot verify an individual's ongoing eligibility at renewal. Based on the most recent state-reported unwinding data available. Time periods differ by state. Rates are calculated as procedural disenrollments divided by total disenrollments. Several states report unwinding data without information on reason for disenrollment and are not shown in this figure.

SOURCE: KFF Analysis of State Unwinding Dashboards and Monthly Reports Submitted to CMS • [Get the data](#) • [PNG](#)

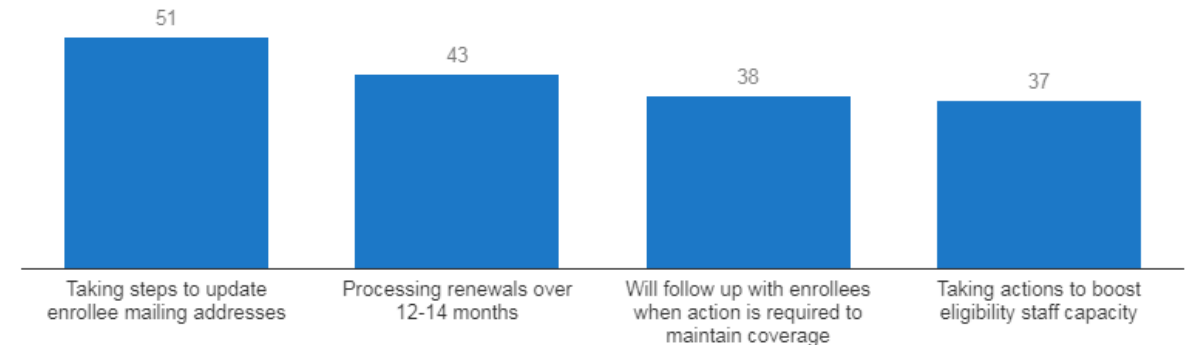
KFF

How have states been approaching Medicaid unwinding?

- State approaches to Medicaid unwinding vary significantly
 - Some states (Arkansas, Iowa, New Hampshire, etc. began the unwinding process in April)
 - Others (Oregon) are holding out until October to begin the process
- 12 states have paused coverage terminations (at least temporarily) due to federal compliance issues related to ex parte renewals
- 10 states have elected to delay procedural terminations for an additional month to complete additional outreach

Figure 4

State Policies and Actions Related to the End of the Medicaid Continuous Enrollment Provision



SOURCE: Based on results from a national survey conducted by KFF and the Georgetown University for Children and Families, 2023 • PNG

KFF

What can I do?

Protect your coverage

1

1. Check the status of your Medicaid coverage through the Federal Medicaid portal [here](#).
2. If you have lost Medicaid coverage, CMS has published a toolkit to assist in either re-applying for Medicaid or identifying alternative insurance options [here](#).
3. If you want to learn more about the unwinding process in your state, click [here](#).

Stay connected + learn more

2

1. NORD will be hosting a webinar on Thursday, September 14th at 2pm Eastern to discuss the impact of Medicaid Unwinding with a panel of experts. Register for the webinar [here](#).
2. If you cannot attend live, NORD will post the recording and accompanying fact sheets on our website.
3. Keep an eye out for mailings from your state Medicaid agency- this year and moving forward!

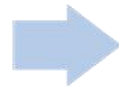
INFLATION REDUCTION ACT IMPLEMENTATION UPDATE

NORD Priorities for IRA Implementation



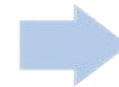
Expand Patient and Provider Input

- Determine value of the therapy and its alternatives
- Evaluate unmet need



Improve Patient Access to Negotiated Drugs

- Place on a higher formulary
- Reduce or eliminate step therapy / prior authorization barriers



Clarify Rare Disease Drug Exemption

- Negotiation eligibility starts at FDA-approval for a second disease or condition

CMS has announced 10 drugs selected for negotiation

10 products selected for negotiation:

- Eliquis
- Jardiance
- Xarelto
- Januvia
- Farxiga
- Entresto
- **Enbrel**
- **Imbruvica**
- Stelara
- Fiasp; Fiasp FlexTouch; Fiasp PenFill; NovoLog; NovoLog FlexPen; NovoLog PenFill

Products in red have an orphan indication

Timeline:

- CMS will meet with companies + host patient listening sessions in Fall, 2023.
- CMS will send an initial offer to the manufacturers of the selected products no later than February 1, 2024. Optionally, manufacturers have 30 days to respond with a counteroffer.
- Counteroffer process will take place during Spring and Summer of 2024. Negotiation process will end August 1, 2024.
- Prices become effective in 2026.

Providing input to the negotiation process

*Want to get involved with the negotiation process? CMS is offering **two ways** for the public to provide input:*

1. Written submissions

- Between September 1 and October 2, 2023, CMS will be soliciting information about the selected drugs on unmet medical need, impacts on specific populations, patient and caregiver experience, and therapeutic alternatives. Information on how to submit comments can be found [here](#).

2. Patient listening sessions

- Between October 30 and November 15, 2023, CMS will host a series of 10 patient listening sessions, one for each of the 10 negotiation eligible products. CMS will randomly select approximately 20 speakers per listening session and each speaker will be limited to three minutes. The calendar for the patient listening sessions can be found [here](#). Register [here](#) for a chance to speak at one of the listening sessions. Registration to speak at one of the listening session will close October 2, 2023.

Background: Difference between designation and indication

- **Early** in drug development
- Intentionally **broad**
- Confers **access to ODA incentives (e.g., FDA research funding, exemption from user fees)**
- **About 6,400** orphan drug designations, many without any associated approved indications
- Designations often **proceed** approved indications by **years or even decades**

Orphan Drug Designation



- **After** clinical trials show drug safe and effective
- **Narrow**, tied to specific evaluated use
- Confers **right to market** the product
- **Approximately 1,100** orphan indications approved by the FDA

FDA Approved Indication



Various orphan designation vs. indication scenarios to explain why a technical fix is needed

Scenario 1: Orphan drug with one orphan designation and one associated indication

**Not
negotiation
eligible**



Scenario 2: Orphan drug with one designations and multiple approved indications, all tied to the same designation

**Not
negotiation
eligible**



Scenario 3: Multiple designations, only one approved indication

**Negotiation
eligible**

Subsequent examples on the following slides are based on FDA's Orphan Drug Designations and Approvals database, accessible at: <https://www.accessdata.fda.gov/scripts/opdlisting/oopd/>

Scenario 1: Orphan drug with one orphan designation and one indication

- **One orphan drug designation (2007):**
 - Treatment of Duchenne Muscular Dystrophy
- **One approved indication (2016):**
 - Treatment of Duchenne Muscular Dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping

Orphan Drugs with one designation & one approved indication are excluded from price negotiation

Designation: Duchenne Muscular Dystrophy (2007)

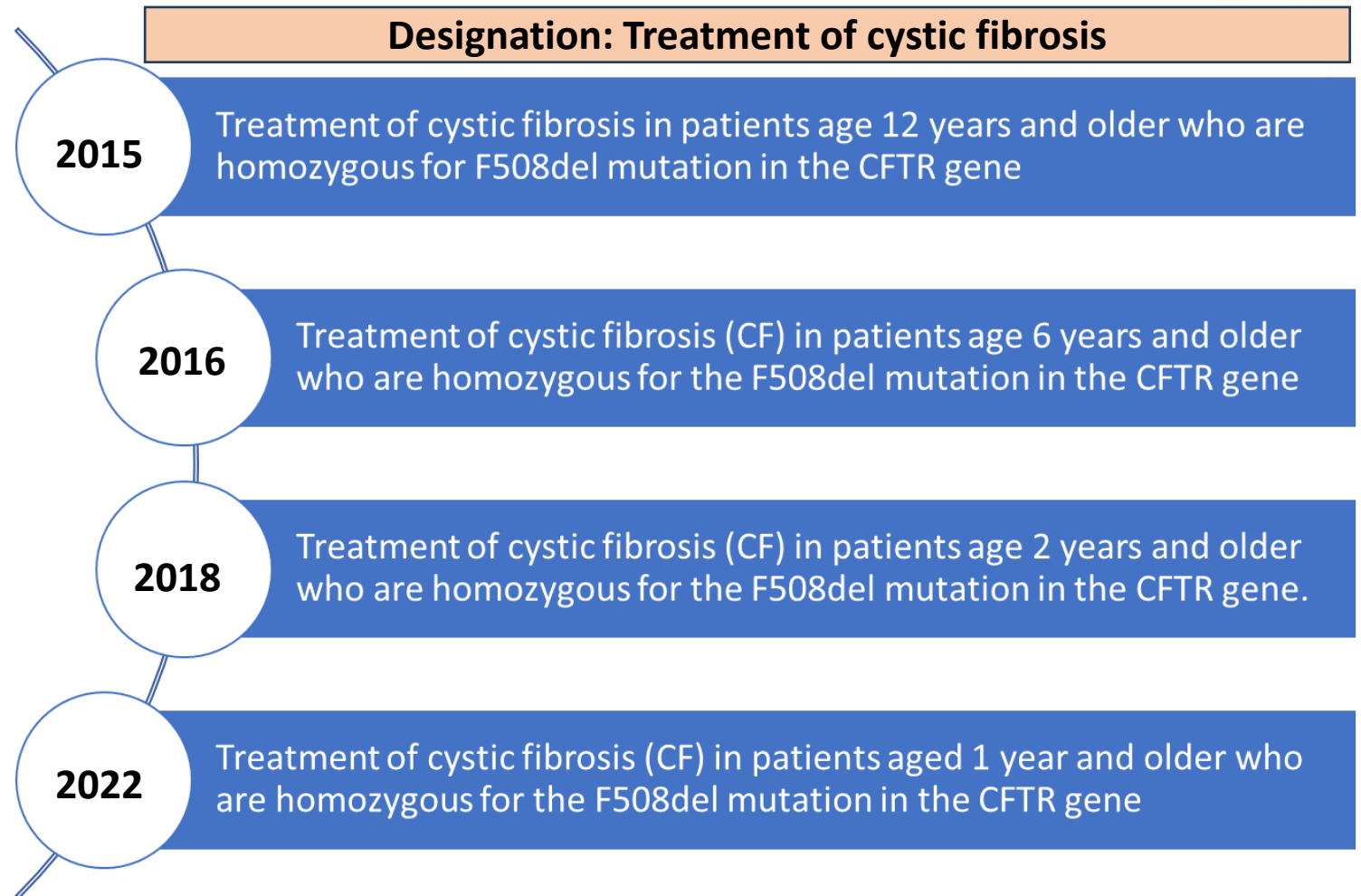
2016

Treatment of Duchenne Muscular Dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping

Scenario 2: Orphan drug with one orphan designation and multiple indications, all tied to the same designation

- **One orphan drug designation (2014):**
 - **Treatment of cystic fibrosis**
- **Four approved indications (2015 – 2022):**
 - **First approved indication in 2015**
 - **Expansion to additional age groups in subsequent years**
- **Incentives to further develop product after market entry help ensure a product is safe and effective for specific populations**

CMS has clarified that products in this scenario are not negotiation eligible



Scenario 3: Orphan drug with multiple designations and only one approved indication

- **Five orphan drug designations (2007 - 2019):**
 - **First designation in 2007**
 - **Subsequent designations starting in 2009, and as recent as 2019**
- **Only one approved indication (2017)**
- **Designations open access to incentives to further develop product in new diseases, which is vital for rare disease patients who often have no FDA approved treatment for their condition**

CMS has clarified that products in this scenario are negotiation eligible because the drug has been designated for more than one disease.

2007	Treatment of scurvy	<ul style="list-style-type: none">• Approved Indication (2017): Treatment of scurvy in adult and pediatric patients age 5 months and older for whom oral administration is not possible, insufficient or contraindicated
2009	Treatment of Charcot-Marie-Tooth disease type 1A.	<ul style="list-style-type: none">• No approved indications
2016	Treatment of lung dysfunction following lung transplant	<ul style="list-style-type: none">• No approved indications
2018	Treatment of cystic fibrosis	<ul style="list-style-type: none">• No approved indications
2019	Treatment of Fragile X syndrome	<ul style="list-style-type: none">• No approved indications

Scenario 4: Orphan drug with multiple designations but only one approved indication

- **Three orphan drug designations (2013 - 2020):**
 - After two designations in 2013, one new orphan designation in 2020
- **Only one approved indication (2021)**
- Designations open access to incentives to further develop product in new diseases, which is vital for rare disease patients who often have no FDA approved treatment for their condition

CMS has clarified that products in this scenario are negotiation eligible because the drug has been designated for more than one disease.

2013

Treatment of alagille syndrome

- Approved Indication (2021): Treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) 1 year of age and older

2013

Treatment of progressive familial intrahepatic cholestasis

- No approved indications

2020

Treatment of biliary atresia

- No approved indications

Background: Few orphan drugs have more than one approved indication & a many products with >1 approval benefited from expedited programs

Table 2. Follow-On Indications for Novel Orphan Drug Approvals

Characteristic	Expedited program approvals, No. (%)		
	Total follow-on indications (N = 152)	Orphan follow-on indications (n = 92)	Nonorphan follow-on indications (n = 60)
Time to follow-on, mean (SD), mo	53.1 (42.5)	56.8 (45.1)	47.3 (37.7)
Time to follow-on, median (IQR), mo	46.4 (18.0-75.3)	48.0 (25.4-85.2)	38.8 (2.7-72.0)
Total expedited programs per approved drug			
None	31 (20)	20 (22)	11 (18)
1	58 (38)	34 (37)	24 (40)
2	46 (30)	29 (32)	17 (28)
3	17 (11)	9 (10)	8 (13)
4	0	0	0
Expedited review program			
Priority	89 (59)	57 (62)	32 (53)
Fast-track	17 (11)	9 (10)	8 (13)
Accelerated	42 (27)	23 (25)	19 (32)
Breakthrough ^a	53 (38)	30 (35)	23 (43)

Source: Chambers JD, Clifford KA, Enright DE, Neumann PJ. Follow-On Indications for Orphan Drugs Related to the Inflation Reduction Act. *JAMA Netw Open*. 2023;6(8):e2329006. doi:10.1001/jamanetworkopen.2023.29006

NORD recommendations for clarifications to the Orphan Drug Exclusion of the Inflation Reduction Act (IRA)

Recommendation 1: FDA designations

Products should only become negotiation eligible after they have been **approved** for more than one disease.

A **designation** unlocks vital Orphan Drug Act (ODA) incentives supporting clinical research and usually occurs early in the drug development process. Many designations never lead to an approved product; current, FDA has approved about 6,400 designations and 1,100 indications. Drug sponsors cannot market the drug until it has been FDA-approved for that **indication**. Tying negotiation eligibility to a designation rather than indication will disincentivize rare disease research.¹

Recommendation 2: When the clock starts

Orphan products should become negotiation eligible 9 or 13 years (depending on type of product) from the date of the FDA approval that **makes them negotiation-eligible**, rather than from the first FDA approval.

On average, it takes about 4.5 years of additional research for an orphan drug to gain a second approval, and >75% of orphan drugs have a single approved indication.²

QUESTIONS?

Thank you.

