2018

NORD®
National Organization
for Rare Disorders

STATE
REPORT
CARD

NORD: The Independent Voice of the Rare Disease Patient Community

The National Organization for Rare Disorders (NORD) is an independent, nonpartisan, nonprofit advocacy organization and the voice of the rare disease patient community. NORD represents the 30 million Americans with rare diseases. We address complex medical, research, and public policy issues through programs and services shaped by a single guiding vision: to improve the lives of all Americans affected by rare diseases.

Since 1983, NORD has ensured that the rare disease patient has had a seat at the table and had his/her voice heard when important federal policy and regulatory decisions are made. Our advocacy began when a group of parents of children with rare diseases came together to advocate for the passage of the Orphan Drug Act of 1983 (ODA). This legislation is regarded as one of the most successful pieces of legislation ever passed by Congress. It was intended to stimulate the research and development of new therapies for rare diseases, which were generally neglected by the research community and the drug industry. Since 1983, more than 600 new drugs to treat rare diseases have been approved by the U.S. Food and Drug Administration (FDA). Many new drugs are now in development, and the outlook for people with rare diseases continues to get brighter.

Following the passage of the ODA, these parent advocates decided there was more work to be done to address the unmet needs of people with rare diseases. As a result, NORD was formed as a mission-based, non-governmental organization. We operate under the slogan that, "Alone we are rare. Together we are strong®." We strive to bring the rare disease community together to raise awareness, educate, empower patients and the organizations that serve them, create a supportive community, and foster collaboration among the various stakeholders who each have a part in driving progress in the fight against rare diseases. Learn more about our work over the past 33 years here: rarediseases.org/history.

In 2010, the implementation of the Affordable Care Act (ACA) addressed some of the issues that were most challenging for people with rare diseases, such as the ability of patients with pre-existing conditions to obtain insurance without lifetime caps on coverage. Many of the patients that we represent have benefited from the ACA, though, at the same time, we know it has not worked for all. Many individuals with rare diseases continue to face barriers to accessing the care and treatment that they desperately need.

In 2015 NORD launched the first-ever ***State Report Card*** to evaluate how states are serving people with rare diseases. We are pleased to present the third edition to demonstrate where progress has been made and where it is still needed. The current political climate poses certain challenges for NORD and the rare disease community. We will continue to work with the current Administration and Congress to best serve the patients whom we represent.

Now, more than ever, we must band together to ensure that the advances we have seen in recent years are not turned back. NORD intends to lead and educate advocates as well as state and federal legislators to protect access to innovative and affordable care for rare disease patients. The actions we take together will have an impact on the lives of so many people. Thank you for your support and for joining us to be a part of this progress.

NORD Mission Statement

The National Organization for Rare Disorders (NORD) is a unique federation of voluntary health organizations dedicated to helping people with rare orphan diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment and cure of rare disorders through programs of advocacy, education, patient/family services and research. www.rarediseases.org

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Introduction

NORD examines public policy using a simple framework: What are the greatest challenges facing rare disease patients and their caregivers at any given stage of life? If you are reading this report, you likely already know what many of these challenges are (or have personally experienced them). These are issues such as the inability to obtain an accurate diagnosis, the high cost of care and treatment, or the social isolation that comes with having such a rare condition.

Our annual ***State Report Card*** seeks to put these obstacles within the context of actions that can be taken to remedy them. For example, what is being done to improve routine screening for detectable diseases, how can states ensure care and treatment is more affordable, and how do we bring individuals with a rare disease together and give them a greater voice in their government?

In this third edition of ***NORD's State Report Card,*** we are seeking to expand our analysis to more issues that affect the rare disease community and provide a more targeted analysis of policies analyzed in previous editions of the report. In terms of the expansion, the 2018 report has grown to cover several emerging issues, such as the enactment of work requirements within Medicaid programs and state adoption of "Right to Try" laws. Here's the list of new issues covered in the 2018 Report Card:

- Medicaid 1115 Waivers (including proposed work requirements, lifetime limits, drug formulary restrictions, and other proposed changes to benefits)
- Storage and research consent for dried blood spot samples used in newborn screening
- State Right to Try laws

In updating state action on the policies from previous editions of this report, NORD has enhanced our analysis of the following issues:

- Coverage of Medical Foods now includes state requirements for a broader array of applicable disorders, including eosinophilic disorders and FPIES
- State newborn screening policies now include analysis of procedures for adding new conditions to the panel of tests (such as whether they require pilot testing and whether there is an official timeline for adopting federal recommendations)
- State action on limiting out-of-pocket costs for prescription drugs now includes policies that require a subset of plans to have a copay-only model

LIST OF POLICIES COVERED IN THIS REPORT

In total, ***NORD's 2018 State Report Card*** now provides detailed analysis on 13 different policy issues:

1. Medical foods coverage requirements for commercial health plans

2. Medical foods coverage requirements for state-run programs

3. Capitation of prescription drug cost sharing in commercial health plans

4. Adoption of federally-recommended (RUSP) newborn screening Core conditions

5. Adoption of RUSP newborn screening Secondary conditions

6. State procedures for adding new diseases to its newborn screening panel

7. Rules on the storage and research uses of newborn screening dried blood spots

8. Medicaid eligibility levels, including for the Children's Health Insurance Program (CHIP)

9. Medicaid 1115 waivers for work requirements and formulary exclusions

10. Biosimilar prescriber communication

11. Patient protections against Step Therapy (fail first) protocols

12. Establishment of Rare Disease Advisory Councils

13. Right to Try requirements

The ***Overview*** section shows the grades states earned for each policy category. However, this year the rest of the results are provided a little differently. Instead of providing an analysis of state performance in each chapter of this report, NORD will be releasing individual state report cards that can be found at rareaction.org. This will make it easier for rare disease advocates and other stakeholders to quickly analyze their state and share that information with others.

Introduction (continued)

METHODOLOGY

Each year, NORD has tried to make our analysis of state policies easier to understand and evaluate, while also being fair to states in how we grade them. For 2018, our grading is simple and straightforward: each policy is graded on an A, B, C, D, F scale. As with NORD's previous State Report Cards, there is no overall grade for a state – only a grade for each policy category. This was done in order to ensure that insufficient state progress in one area would not unfairly skew perception of other policy areas where a state is excelling (and vice versa).

While the interpretation of state grades for individual policies will obviously vary, the following is a general analysis of how NORD views each grade:

A: State policy meets all desired standards. An A grade is considered model policy that other states should seek to enact

B: State policy meets most, but not all desired standards

C: State policy on the given issue meets minimum standards

D: State has some policy in place, but it does not meet the standards of higher tiers

F: State has no policy provision for the relevant issue

See each individual section of this report for the exact rubric used for every policy and additional information for how we evaluated the states.

USING THE APPENDICES AND INDIVIDUAL STATE REPORT CARDS

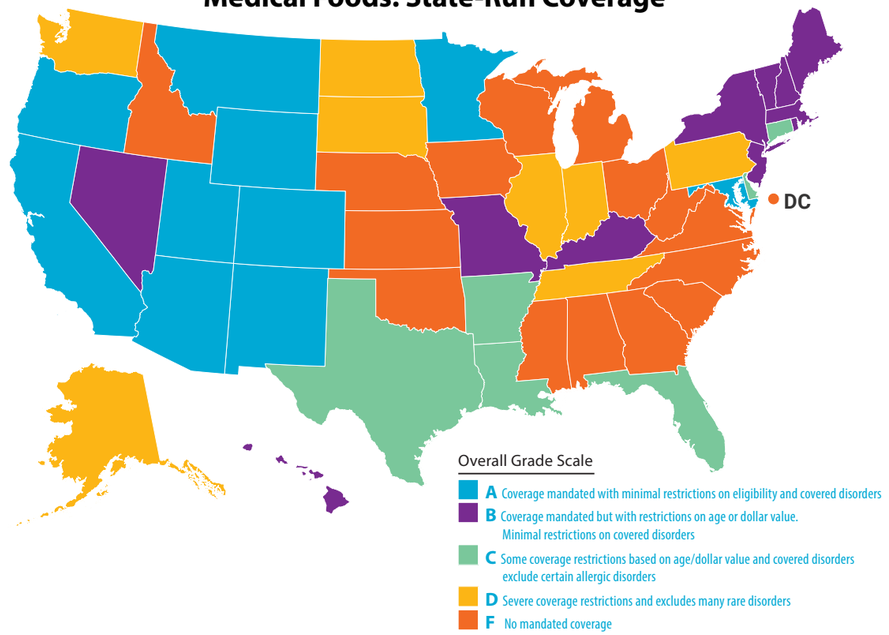
This write-up of the State Report Card provides important background on each policy issue we analyzed, as well as information about our grading methodology. However, the bulk of the information about each state can be found in our appendices. There is an appendix for each category of this report, and it provides all of the information used to determine grades, such as specific state statutes and eligibility for certain programs. In reading this entire report, we encourage you to look beyond the topline grades for each category and use the appendices to learn more about your state's policies.

Finally, for 2018, NORD will be releasing individual report cards for each state. As noted previously, this will make it easier for stakeholders to quickly see the grades for their state and share it with others. These individual state reports will not include information on specific state statues included in the appendix.

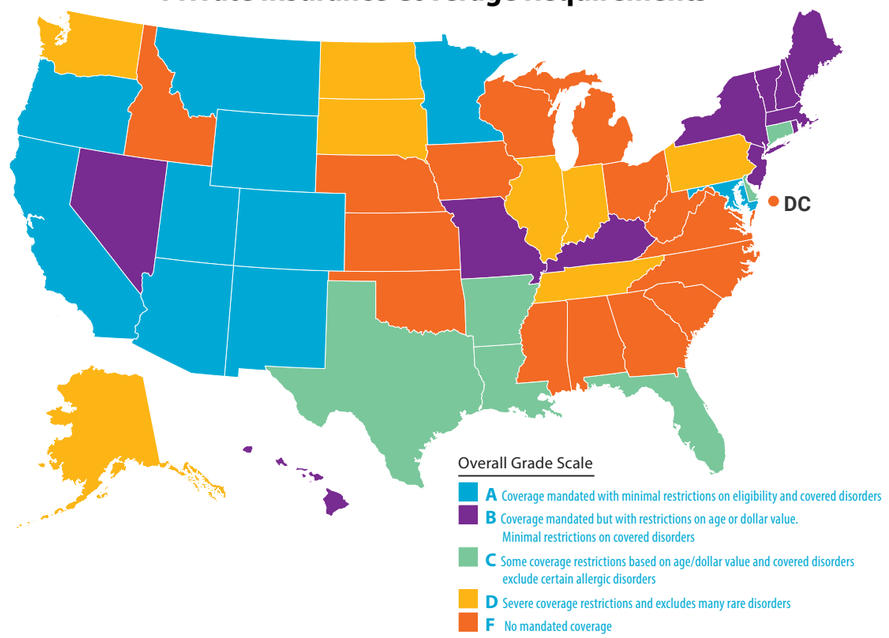
SECTION I
NATIONAL OVERVIEW

National Overview

Medical Foods: State-Run Coverage

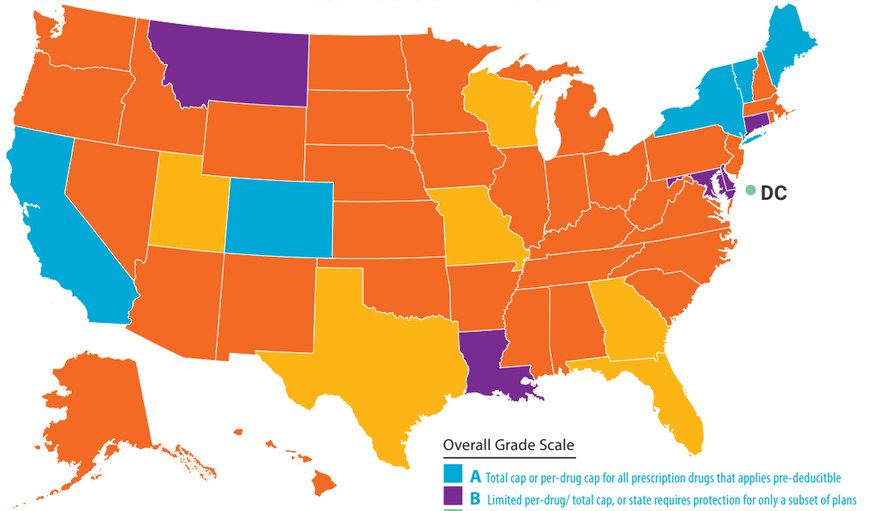


Medical Foods: Private Insurance Coverage Requirements



National Overview (continued)

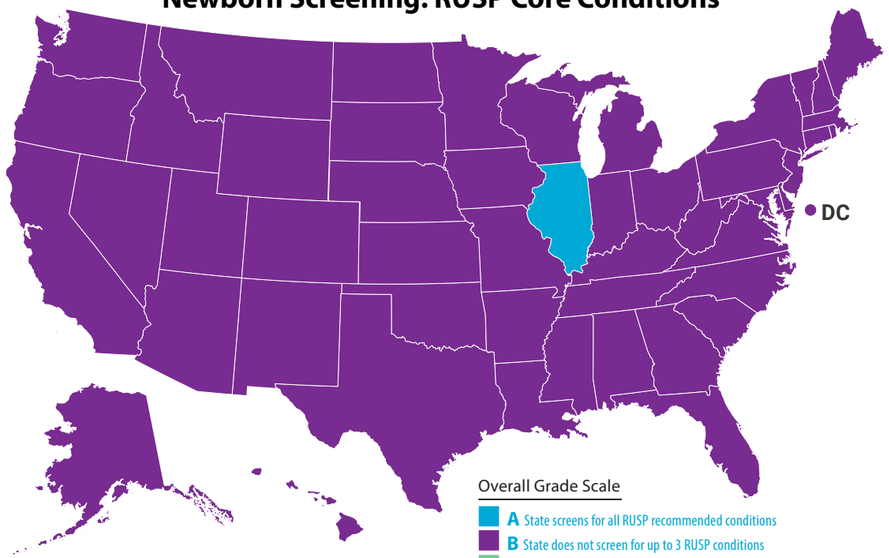
Rx OOP Protections



Overall Grade Scale

- **A** Total cap or per-drug cap for all prescription drugs that applies pre-deducitble
- **B** Limited per-drug/ total cap, or state requires protection for only a subset of plans
- **C** Per drug or total cap do not apply pre-deductible and may not cover all coinsurance
- **D** Cost sharing limits for only a limited class of drugs
- **F** No policy

Newborn Screening: RUSP Core Conditions

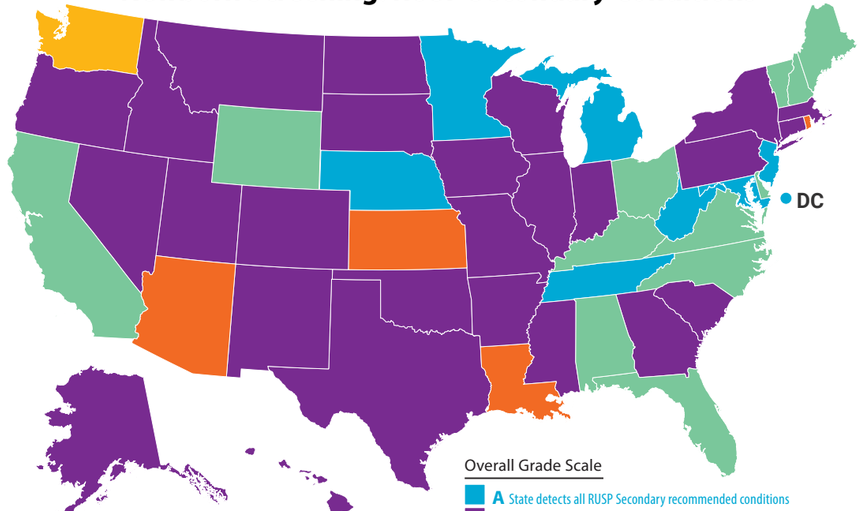


Overall Grade Scale

- **A** State screens for all RUSP recommended conditions
- **B** State does not screen for up to 3 RUSP conditions
- **C** State does not screen for 4 to 5 RUSP conditions
- **D** State does not screen for more than 5 RUSP conditions
- **F** N/A

National Overview (continued)

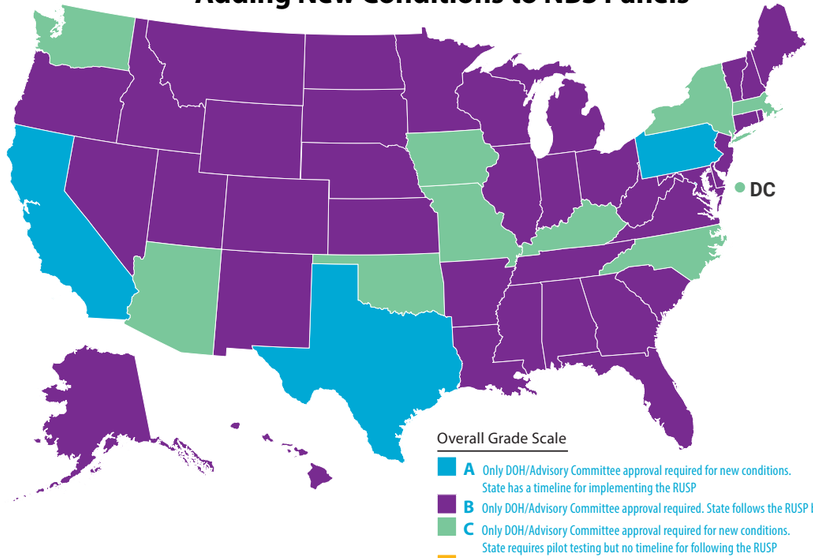
Newborn Screening: RUSP Secondary Conditions



Overall Grade Scale

- **A** State detects all RUSP Secondary recommended conditions
- **B** State omits detection of up to 5 Secondary recommended conditions
- **C** State omits detection of 6 to 10 Secondary recommended conditions
- **D** State omits detection of 11 to 15 Secondary recommended conditions
- **F** State omits detection of more than 15 Secondary recommended conditions

Adding New Conditions to NBS Panels

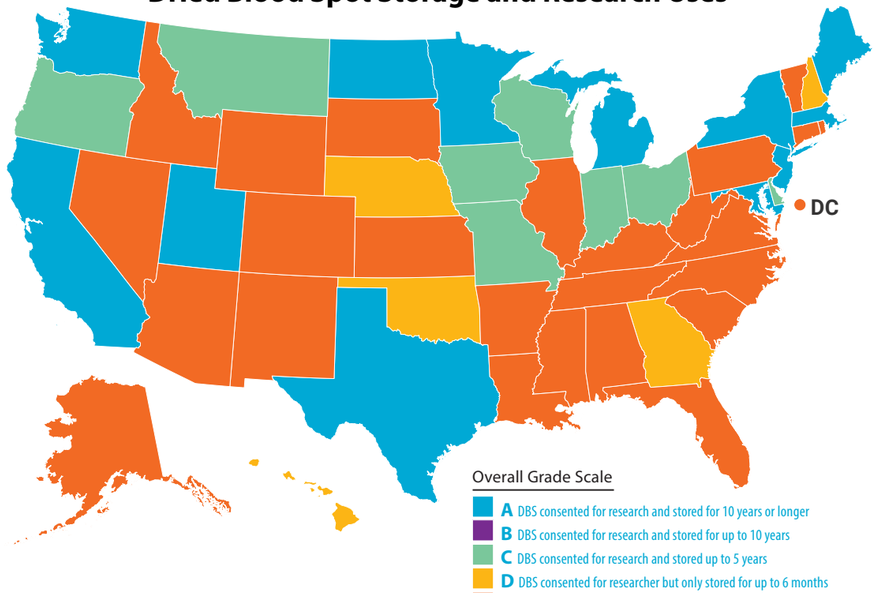


Overall Grade Scale

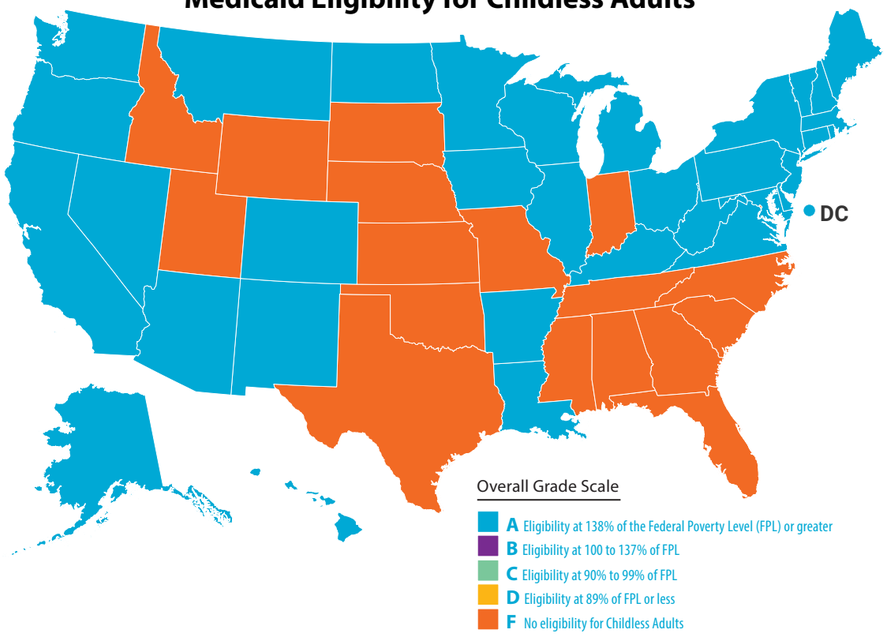
- **A** Only DOH/Advisory Committee approval required for new conditions. State has a timeline for implementing the RUSP
- **B** Only DOH/Advisory Committee approval required. State follows the RUSP but no timeline
- **C** Only DOH/Advisory Committee approval required for new conditions. State requires pilot testing but no timeline for following the RUSP
- **D** Legislative approval required
- **F** N/A

National Overview (continued)

Dried Blood Spot Storage and Research Uses

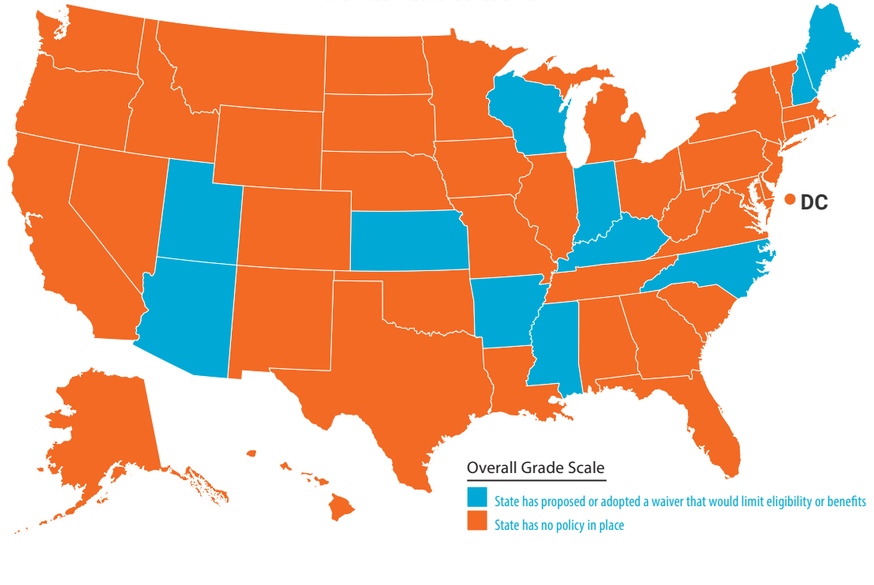


Medicaid Eligibility for Childless Adults

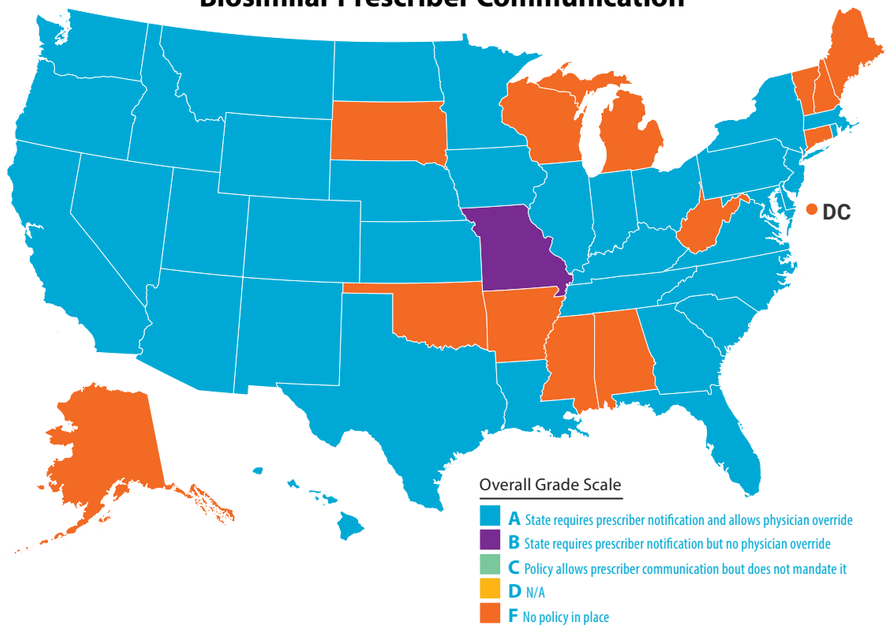


National Overview (continued)

Medicaid Waivers

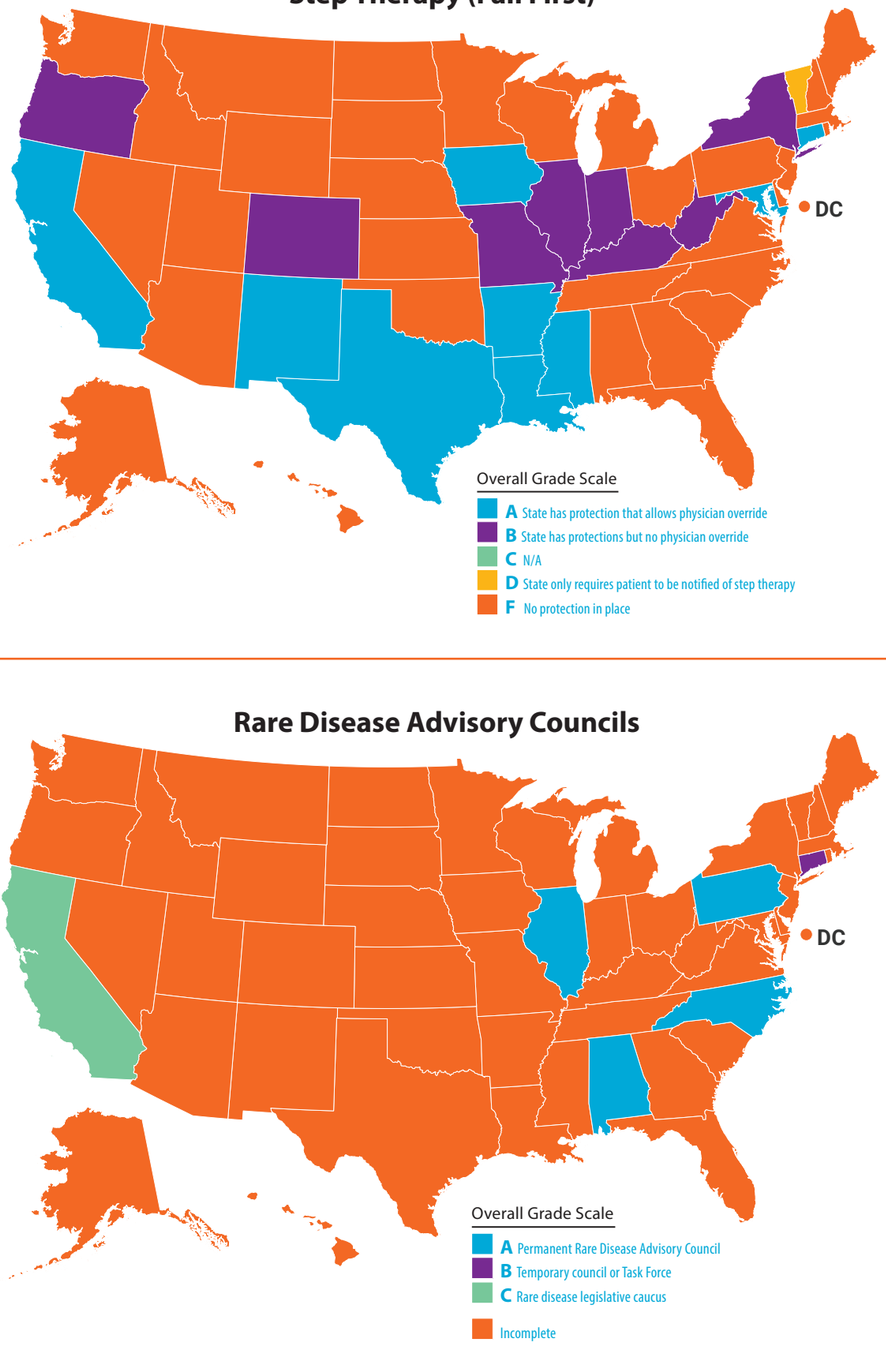


Biosimilar Prescriber Communication



National Overview (continued)

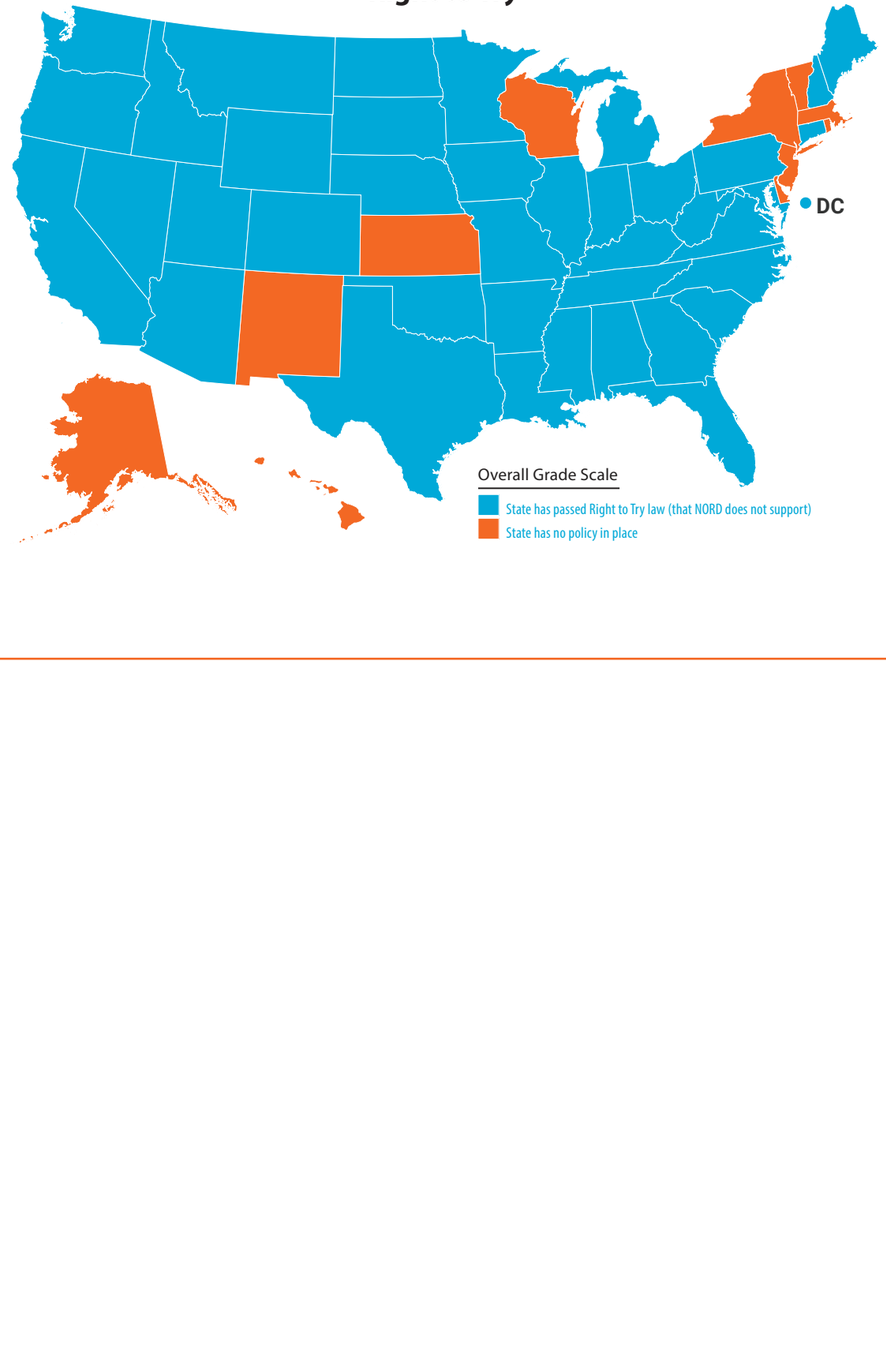
Step Therapy (Fail First)



Rare Disease Advisory Councils

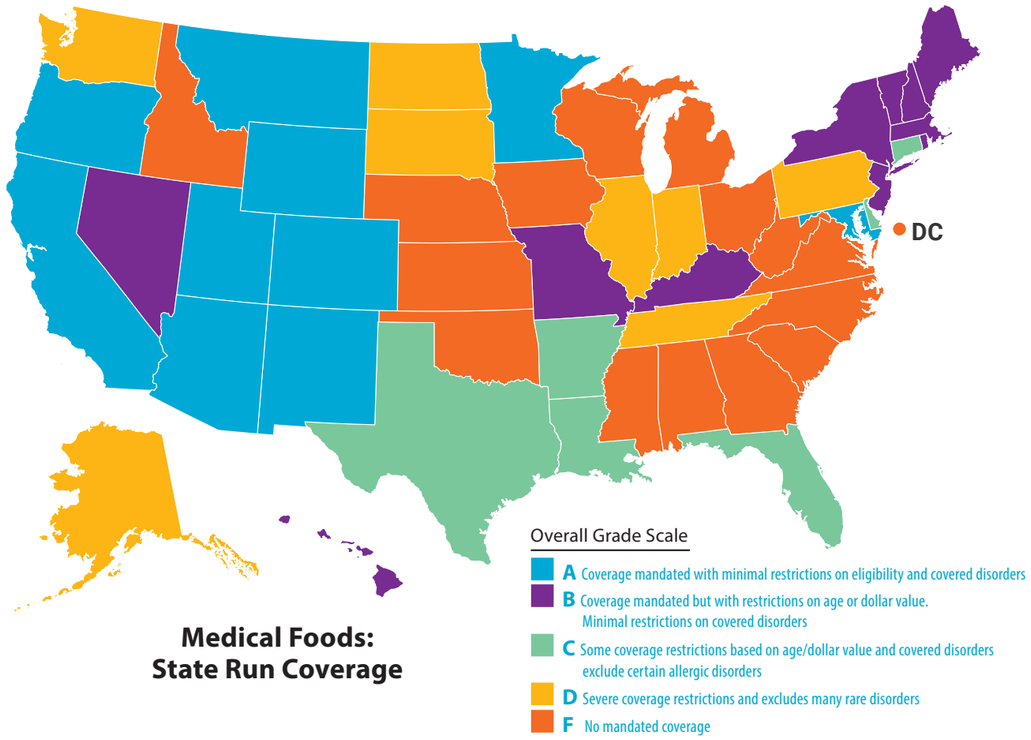
National Overview (continued)

Right to Try



SECTION II
EXPLORING THE ISSUES

Medical Foods



Medical Foods: State Run Coverage

STATE COVERAGE REQUIREMENTS FOR COMMERCIAL HEALTH PLANS

There are multiple rare disorders that require special nutrition in order to prevent serious disability and allow for normal growth in children and adults. For patients living with these conditions, effective medical foods are the only viable treatment option available.

The manufacture of these medical foods is highly specialized, making them more expensive for patients. For example, the average annual cost of formula for the metabolic disorder PKU (phenylketonuria) can cost as much as $12,000[1]. Third-party payment for foods for special dietary use is inconsistent, and state statutes regarding reimbursement vary widely.

Some states require coverage only for inherited metabolic diseases, such as PKU, and others include a range of metabolic conditions. While much can be done at the federal level to increase access to medical foods, states also play an integral role in ensuring access to these critical therapies.

Because insurance is regulated primarily at the state level, many states have mandated the inclusion of medical foods within private plans sold within their state. However, in the states that do not have medical food mandates, individuals in need of these particular treatments are faced with a huge burden of access and require assistance in paying for medical food expenses.

Medical Foods

COVERAGE REQUIREMENTS FOR STATE-RUN PROGRAMS

Coverage of medical foods within each state's Medicaid program is also essential, yet only some states mandate coverage. For states that do mandate coverage through Medicaid, a few have chosen to provide access to medical foods through other publicly-funded health programs or provide coverage on a case-by-case basis (which can lead to high variability in which patients have access).

While mandating coverage of medical foods in states is a big step forward, too many states place arbitrary cost, age, or gender limits on these coverage requirements. NORD encourages each state to adopt coverage mandates for medical foods without arbitrary limitations.

COVERED DISORDERS

Who is eligible for Medicaid foods coverage is just as important as what kind of coverage they will receive. Unfortunately, many states limit coverage (either in commercial insurance mandates or in Medicaid) to certain disorders. Traditionally, most states have focused their coverage on metabolic conditions and have expanded eligibility to a variety of such disorders.

More recently, however, states have begun to expand coverage for other conditions that require specialized nutrition. Many of these disorders are allergic in nature (symptoms are caused by the body's reaction to certain food ingredients) and can be misconstrued as simple food allergies that can be easily avoided. In truth, disorders such as Eosinophilic Esophagitis or Food Protein-Induced Enterocolitis Syndrome (FPIES) require highly specialized nutritional products in order to be properly treated. In the ***2018 State Report Card,*** NORD included these disorders in its analysis and supports Medical Foods coverage for any condition for which it is a medically necessary component of effective treatment.

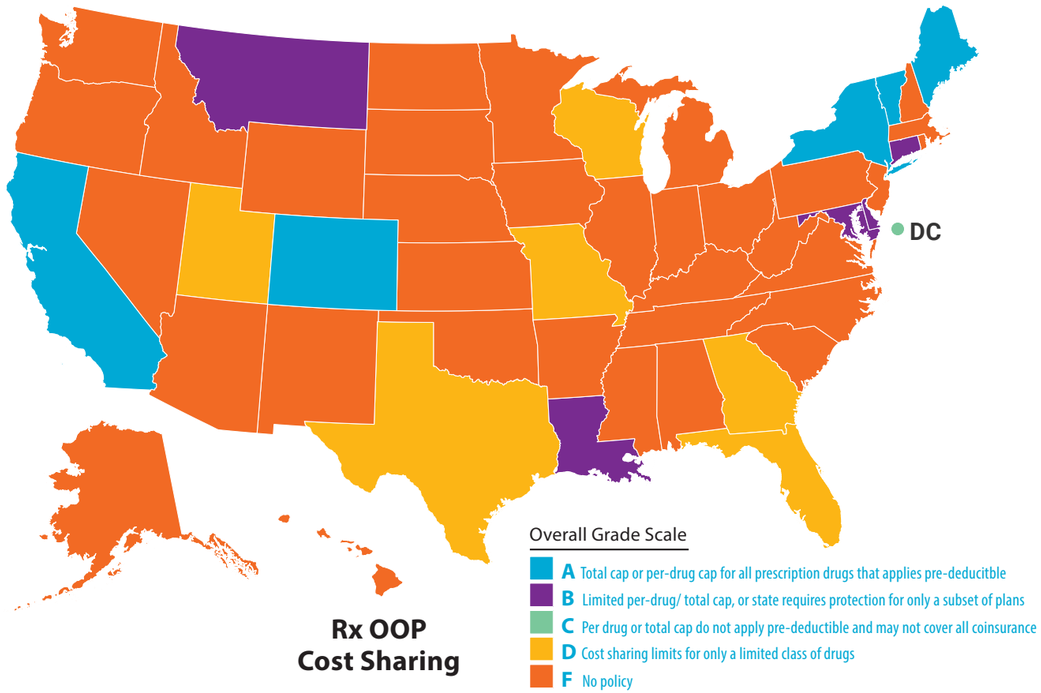
METHODOLOGY

The grading rubric for Medical foods can be found on page 17. States were graded separately on two policies: coverage requirements for commercial health plans (including covered disorders) and coverage mandates for state-run programs (also including covered disorders). States that placed age or dollar amount restrictions on coverage earned lower grades than states that had no such restrictions. Similarly, states with more covered conditions (ideally, any condition with a medically necessity for medical foods) graded better than states with fewer. In addition, as previously mentioned, NORD marked states down if they did include eosinophilic disorders or FPIES in their covered conditions.

Table 1: Medical Foods Grading Rubric

	DESCRIPTION	
GRADE	**Private Insurance Coverage Requirements (Including Covered Disorders)**	**State-Run Coverage**
A	Coverage is required for both formula and low-protein foods with no limits on eligibility or coverage. Covered disorders include all inborn errors of metabolism; eosinophilic disorders/ FPIES; or medically necessary treatment.	Mandated Medicaid coverage for medical foods with no age or eligibility restrictions (or through a supplemental program). Covered disorders include all inborn errors of metabolism; eosinophilic disorders/ FPIES; or medically necessary treatment.
B	Coverage is required for formula and low-protein food but with age or dollar limits. Covered disorders include all inborn errors of metabolism; eosinophilic disorders/ FPIES; or medically necessary treatment.	Mandated Medicaid coverage for formula and low-protein foods (or through a supplemental program) with restrictions. Covered disorders include all inborn errors of metabolism; eosinophilic disorders/ FPIES; or medically necessary treatment.
C	Coverage is required for both formula and low-protein foods, but with age and dollar limits. Covered disorders do not include eosinophilic disorders, FPIES, and other medically necessary uses.	Coverage for formula and low-protein foods is on a case-by-case basis. Covered disorders do not include eosinophilic disorders, FPIES, and other medically necessary uses.
D	Coverage required but with limits on eligibility (such as age) or coverage (such as a dollar cap or formula only). Covered disorders include 3 or fewer metabolic conditions (such as PKU-only) and do not include eosinophilic disorders, FPIES, and other medically necessary uses.	Mandated Medicaid coverage for formula but no coverage of low-protein foods. Covered disorders include two or fewer metabolic conditions (such as PKU-only) and do not include eosinophilic disorders, FPIES, and other medically necessary uses.
F	State does not mandate private insurance coverage of medical foods.	State does not mandate coverage for Medicaid. The state does not offer supplemental programs to provide coverage.

Prescription Drug Cost Sharing



BACKGROUND

Thanks to innovative new treatments, diseases that were once fatal are now being treated as chronic conditions. But these breakthrough treatments will be out of reach for many patients because health plans are using deductibles and coinsurance to shift more of the cost of medication onto the patients who rely on those treatments. Together, those out-of-pocket costs are outpacing wages, and patients are left struggling.

For example, plans often require that enrollees pay co-payments each time they fill their prescription that can be as much as 50% of the actual cost of the medication.[1] For many people with a rare disorder, as well as those with other severe chronic diseases, these costs are untenable. As a consequence, patients in need of life saving treatment are forced to go without their medication or use options that are less effective and less safe.

The utilization of this type of cost-sharing structure in health plans is staggering: In 2017, 84% of Silver Plans (the most common type of health insurance plan on the individual market) had a coinsurance requirement for so-called specialty drugs.[2]

To assist patients who find themselves in this difficult situation, several states have passed legislation mandating a

Prescription Drug Cost Sharing

limit on out-of-pocket costs for specialty medications. These limits range from $100 to $500 per-month per-medication, depending on the type of plan. NORD strongly supports the enactment of these types of policies as they greatly benefit rare patients at a minimal impact to the overall insured population. In fact, third-party analysis has demonstrated that these types of limits on co-pays can be instituted with little to no impact on overall plan premiums for all beneficiaries.[3]

In addition, NORD is now supporting policy models which do not apply per-drug caps on out-of-pocket costs, but rather require that patients have a choice for a "copay only" model when choosing a plan. Under this model, each insurance carrier must ensure that a pre-deductible copay is applied to the entire prescription drug benefit in at least 25% of individual and group plans offered in each service area and on each metal tier. This copay-only benefit design must be reasonably graduated and proportionately related across all tiers of the plan's formulary.

This proposal is also highly feasible to implement. Legislation based on a "copay-only" rule has already been adopted by Colorado's insurance commissioner in 2015.[4] That rule required insurers to offer at least some plans that feature more affordable drug coverage. In that state, patients now have different types of plans to choose from, including some that use only copays in the drug benefit, and they do not have to pay significantly higher premiums for one of these copay-only plans than for other kinds of plans.

METHODOLOGY

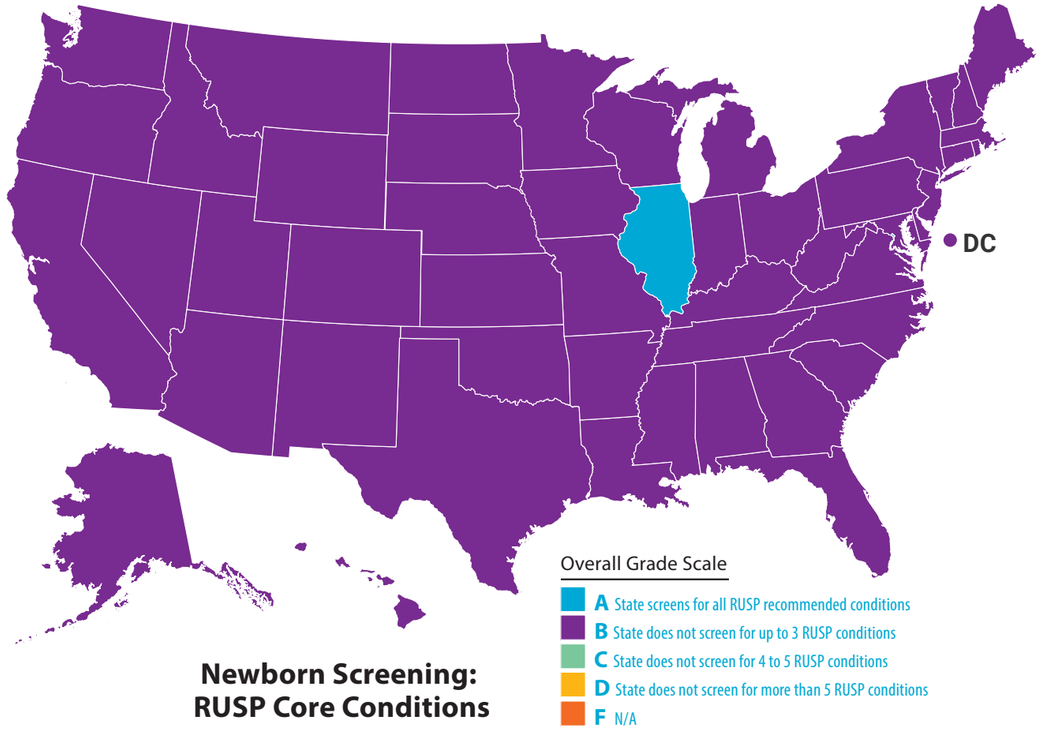
When it comes to addressing the issue of high drug cost sharing, there are several different policies states can implement that are effective. For example, some states have chosen to limit co-pays on a per-drug, per-month basis. Others have mandated total caps for all drug cost sharing. Finally, a few states have implemented (or are considering) the copay only model.

The entire grading rubric for this section can be found on page 19. In general, NORD graded states higher if they had stronger protections (per drug caps) that applied to all classes of drugs. States earned an A grade if they instituted a total cap or per-drug cap on prescription drug cost sharing that applies pre-deductible and to all prescription drugs. States earned a B if they instituted a per-drug cap or total cap on cost sharing that applies pre-deductible and only applies to specialty-tier drugs, or they require 25% of eligible plans to offer a co-pay only benefit for prescription drugs.

Table 2: Prescription Drug Cost Sharing Grading Rubric

	DESCRIPTION
GRADE	**Rx Cost Sharing Grading Rubric**
A	State has instituted a total cap on or per-drug cap on Rx cost sharing that applies pre-deductible and for all prescription drugs.
B	State has instituted a per-drug cap or total on cost sharing that applies pre-deductible. Cap only applies to specialty-tier drugs. OR State requires 25% of eligible plans to offer a co-pay only benefit for prescription drugs.
C	State has instituted a per-drug cap or total cap on cost sharing that does not apply pre-deductible.
D	State has enacted cost sharing limits for a limited number of treatments (such as oral chemotherapy only).
F	State does not have a cap on cost sharing.

Newborn Screening



BACKGROUND

Newborn screening (NBS) is one of the most successful public health programs ever enacted, saving thousands of lives over the past 50 years. Newborn screening allows physicians to catch a heritable disease early and start treatment almost immediately following birth. In this way, many of the worst effects of a disease can be mitigated.

Newborn screening programs are regulated and operated almost entirely at the state level, allowing customization of the program to the state's specific needs. For example, states have great leeway in terms of what conditions to screen for and how samples are used following a blood spot test.

NORD supports robust, well-funded newborn screening programs in every state. We also encourage state lawmakers to work with their health department to prioritize the early detection of these debilitating diseases. NORD encourages every state to adopt the Uniform Newborn Screening Panel developed by the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children[1] and will continue to advocate for this adoption in each state that currently does not screen for the disorders included within the panel.

Newborn Screening

STATE PROCESSES FOR ADDING NEW CONDITIONS

We recognize that the strength of any state's NBS program is not limited to the number of conditions detected. State government support for its NBS labs (including funding for personnel and new tests) is critically important, as is the process by which states can add new conditions to its program. These issues can distinguish a state from others above and beyond whether specific conditions are on its NBS panel (and whether every condition is mandated for all populations). This report has expanded its analysis of NBS to better capture these issues and compare state programs.

STORAGE AND RESEARCH USES OF DRIED BLOOD SPOTS USED IN NBS

The Dried Blood Spot (DBS) dean from a baby's heel shortly after birth (the primary tool of NBS) is an invaluable source of research data on not only the diseases covered by NBS programs, but for a host of other conditions. Most states do not specify how DBS samples will be used in research. However, uses generally fall into three categories. First, DBS is used for quality assurance purposes, such as to verify the results of other NBS tests. Second, states use DBS to advance knowledge and tools for screening itself, such as the development of new tests and improvement of existing testing technology. And finally, DBS is provided to outside researchers to conduct clinical studies on the diseases themselves or to better understand the genetic origins of disease. In some cases, this research can lead to new treatments.

Within these categories of research, the medical breakthroughs achieved through the use DBS are numerous. For example, samples have been used to study the prenatal and genetic origins of leukemia and autism spectrum disorder.[2] Perhaps most famously, DBS was essential to the development of a newborn screening test for Severe Combined Immunodeficiency Disorder (SCID), a debilitating rare disease that can be effectively treated so long as it is identified early enough.[3] The DBS samples used in each of these advancements were obtained from health department archives, where they are stored following screening.

Currently, numerous states do not have a policy for retaining DBS after its use in screening nor for parental consent for it to be used in research. NORD believes that the primary aim of screening, to detect treatable diseases in newborns, can be achieved while also promoting the use of DBS in research. Therefore, we encourage states to adopt policies to store DBS samples for longer periods of time and to solicit informed consent from parents to use these samples in research.

METHODOLOGY

The complete newborn screening grading rubric can be found on page 22. NORD evaluated each state on the following sections:

RUSP CORE CONDITIONS

We analyzed the number of RUSP Core conditions screened for in each state. States were graded the same regardless of whether they implemented universal screening or screening for select populations.

RUSP SECONDARY CONDITIONS

We analyzed the number of RUSP Secondary conditions screened for in each state. States were graded the same regardless of whether they implemented universal screening or screening for select populations. In addition, states were graded the same regardless of whether the Secondary conditions were detected and reported while testing for another condition.

DRIED BLOOD SPOT RESEARCH USES

We analyzed how long states allow DBS to be stored and whether they allow research uses (with parent consent).

STATE PROCESS FOR ADDING NEW CONDITIONS

In examining each stat's process for adding new diseases to its panel, we focused on three key issues: First, does the state require legislative approval for every new condition or can it be added once the Department of Health and NBS Advisory Committee approves it.

Second, does the state require pilot testing for every new condition added to the panel. Pilot testing is an important process by which health departments can evaluate the logistical capacity of their health providers and labs to implement new tests. However, not all states require such pilot testing and, without a formal time frame to implement new tests, it can delay the adoption of RUSP recommendations. Finally, we examined whether states have reported that it is their official policy to adopt RUSP recommendations and whether they have a time frame for doing so (such as within two years).

Table 3: Newborn Screening Grading Rubric

	DESCRIPTION			
GRADE	**Adoption of RUSP Core Conditions**	**Adoption of RUSP Secondary Conditions**	**Process for Adding New Conditions**	**Research Uses of DBS**
A	State screens for all RUSP Core Conditions	State detects all RUSP Secondary Conditions	Only DOH/Advisory Committee approval required. If pilot testing is required, the state has an official timeline for following RUSP recommendations of less than 2 years	State stores DBS samples for 10 years or longer and has a consent policy for research uses
B	State does not screen for up to 3 RUSP Core Conditions	State omits detection of 1 to 5 RUSP Secondary Conditions	Only DOH/Advisory Committee approval required. If pilot testing is required, the state has an official policy for following RUSP recommendations (but no set a time frame for doing so)	State stores DBS samples for up to 10 years and has a consent policy for research uses
C	State does not screen for 4 to 5 RUSP Core Conditions	State omits detection of 6 to 10 RUSP Secondary Conditions	Only DOH/Advisory Committee approval required. Pilot testing of new conditions is required. State does not have an official timeline for following RUSP recommendations	State stores DBS samples for up to 5 years and has a consent policy for research uses
D	State does not screen for more than 5 RUSP Core Conditions	State omits detection of 11 to 15 RUSP Secondary Conditions	New conditions are only added via legislative approval	States stores DBS for up to 6 months and has a consent policy for research uses
F	--	State omits detection of more than 15 RUSP Secondary Conditions	--	State does not allow DBS to be used for research (beyond QA)

Newborn Screening

Table 4: Newborn Screening: RUSP CORE Conditions

NEWBORN SCREENING: RUSP CORE CONDITIONS DEFINITIONS
For more information on the diseases listed, visit NORD's Rare Disease Database or the National Institutes of Health

Hearing	Hearing loss	**HMG**	3-Hydroxy-3-methylglutaryl-CoA lyase deficiency
CH	Congenital hypothyroidism	**IVA**	Isovaleric acidemia
CAH	Congenital adrenal hyperplasia	**3-MCC**	3-Methylcrotonyl-CoA carboxylase deficiency
S/S, S/A, S/C	Sickle cell disease	**Cbl-A,B**	Methylmalonic acidemia
BIO	Biotinidase deficiency	**BKT**	Beta-ketothiolase deficiency
GALT	Galactosemia	**MUT**	Methymalonyl-CoA mutase deficiency
CF	Cystic fibrosis	**PROP**	Propionic acidemia
CCHD	Critical congenital heart defect	**MCD**	Holocarboxylase synthetase deficiency
SCID	Severe combined immunodeficiency	**ASA**	Argininosuccinic aciduria
CUD	Carnitine uptake defect	**CIT**	Citrullinemia, type I
LCHAD	Long-chain 3-hydroxyacyl-CoA dehydrogenase deficiency	**HCY**	Homocystinuria
MCAD	Medium-chain acyl-CoA dehydrogenase (MCAD) deficiency	**MSUD**	Maple syrup urine disease
TFP	Trifunctional protein deficiency	**PKU**	Phenylketonuria
VCLAD	Very long-chain acyl-CoA dehydrogenase deficiency	**TYR-1**	Tyrosinemia, type I
MPS I	Mucopolysaccharidosis Type I	**ALD**	Adrenoleukodystrophy
GA-1	Glutaric acidemia, type 1	**POMPE**	Pompe Disease

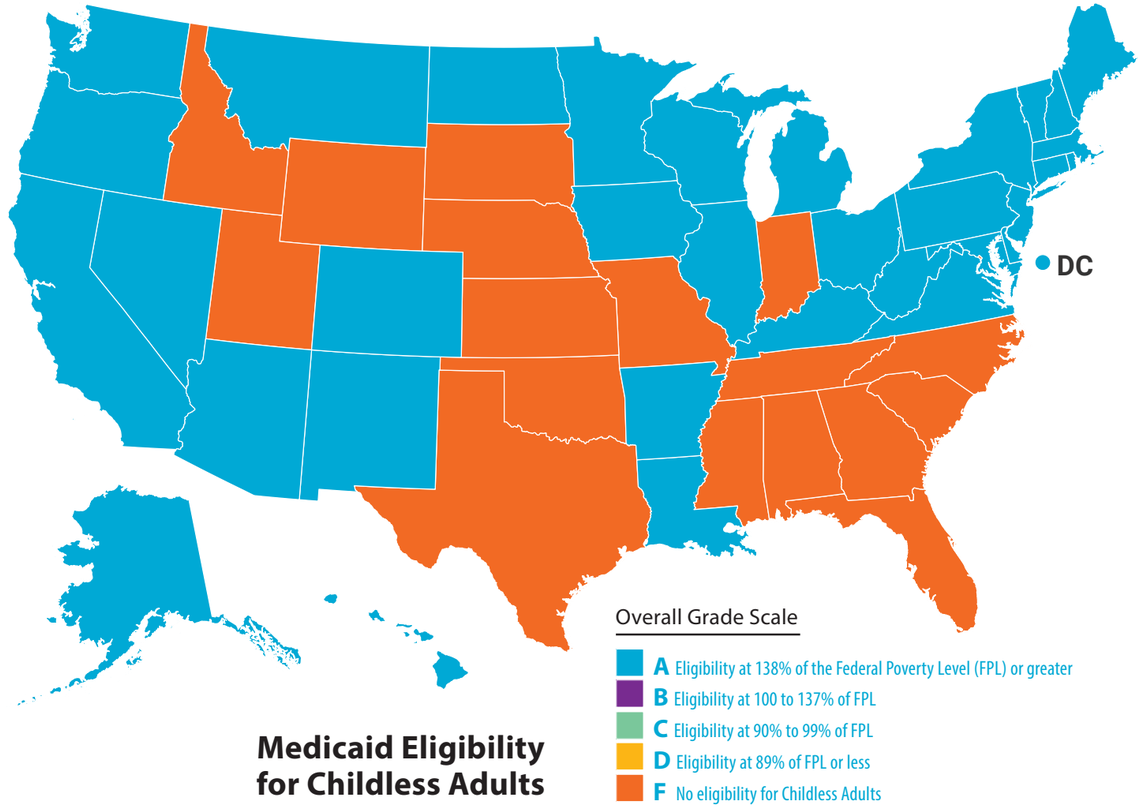
Newborn Screening

Table 5: Newborn Screening Secondary Conditions

NEWBORN SCREENING: SECONDARY CONDITIONS DEFINITIONS
For more information on the diseases listed, visit NORD's Rare Disease Database or the National Institutes of Health

CACT	Carnitine-acylcarnitine translocase deficiency	**IBG**	Isobutyrylglycinuria
CPT-1A	Carnitine palmitoyltransferase 1A	**MAL**	Mal de Meleda
CPT-II	Carnitine palmitoyltransferase II deficiency	**ARG**	Argininemia
DE-RED	2,4 Dienoyl-CoA reductase deficiency	**BIOPT-BS**	Biopterin defect in cofactor biosynthesis
CA-II	Carbonic anhydrase II	**BIOPT-RG**	Biopterin defect in cofactor regeneration
GA 2	Glutaric acidemia type II	**CIT-II**	Citrullinemia, type II
MCKAT	Medium-chain ketoacyl-CoA thiolase deficiency	**H-PHE**	Hyperphenylalaninemia
M/SCHAD	3-Hydroxyacyl-CoA dehydrogenase deficiency	**MET**	Hypermethioninemia
SCAD	Short-chain acyl-CoA dehydrogenase deficiency	**TYR-II**	Tyrosinemia, Type II
2M3HBA	2-Methyl-3-hydroxybutyric academia	**TYR-III**	Tyrosinemia, Type III
2MBG	2-Methylbutyryl-CoA dehydrogenase deficiency	**GALE**	Galactoepimerase deficiency
3MGA	3-Methylglutaconyl-CoA hydratase deficiency	**GALK**	Galactokinase deficiency
CbI-C,D	Cobalamin C cofactor deficiency	**HBS**	HbS disease

Medicaid



Medicaid Eligibility for Childless Adults

BACKGROUND ON MEDICAID FINANCIAL ELIGIBILITY

In 2012, the Supreme Court decision in National Federation of Independent Business v. Sebelius enabled states to choose whether or not to expand the financial eligibility for their Medicaid program. Since the decision, a growing number of states have decided to expand their Medicaid programs to cover all individuals at or below 138 percent of the federal poverty level (FPL). States that have opted not to expand their eligibility have left approximately 5 million Americans without health insurance who would otherwise be eligible for Medicaid coverage. NORD strongly supports expanding Medicaid in every state as it would increase access to needed health services and allow thousands of Americans with rare diseases to gain health insurance coverage.

The State Children's Health Insurance Program (CHIP) is an important source of health coverage for children and families that are ineligible for traditional Medicaid. All states provide increased coverage for children and families through CHIP but may operate the program slightly differently. For example, some states use the federal funding for CHIP to expand their Medicaid program to reach this target population (this is sometimes referred to as "CHIP-funded eligibility"). Other states use these funds to operate a separate CHIP program that provides separate coverage from their Medicaid program.

Medicaid

BACKGROUND ON MEDICAID 1115 WAIVERS

In an attempt to control health care costs and improve services for Medicaid beneficiaries, states have sought Section 1115 waivers that would enable them to make substantial changes to Medicaid benefits and eligibility. We at the National Organization for Rare Disorders (NORD) understand the need for states to consider creative policy solutions to improve health care quality and access. However, we have grave concerns that many of the current state proposals will have a detrimental effect on the rare disease community.

Section 1115 waivers enable the Federal Government to approve state-administered demonstration projects that the Centers for Medicare and Medicaid Services (CMS) determines to be of benefit to the objectives of the Medicaid program. These projects waive certain Medicaid requirements and allow a state to direct federal Medicaid funds in ways that would otherwise not be permitted. If enacted, NORD believes that several of the current proposals would restructure Medicaid benefits and eligibility in a way that undermines the purpose of the program and disproportionately affects people with rare diseases.

Multiple states have proposed to implement "commercial-style" formulary restrictions in Medicaid programs that would limit coverage for new therapies approved by the Food and Drug Administration (FDA). NORD opposes any formulary restrictions that cut access to vital orphan therapies. Restricting drug benefits would limit the ability of providers to make the best medical decisions for their patients. This could inordinately affect rare disease patients because they disproportionately rely on the new and innovative medicines these states are aiming to restrict. Rare disease patients deserve the same access to life-improving, even life-saving, medications as everyone else, and these harmful proposes will disproportionately impact rare disease patients and their families.

In addition, many states are proposing to add work requirements to their Medicaid programs. On January 11, CMS released a letter to Medicaid Directors signaling its support for work requirements. The following day, CMS approved Kentucky's 1115 waiver, the first approved waiver to include work requirements.[1]

NORD opposes the implementation of work requirements, as we believe the exemptions to these requirements will not adequately address the complex health challenges facing rare disease patients. With a scarcity of physicians familiar with rare diseases and the prevalence of undiagnosed conditions, it is often difficult, even impossible, for rare disease patients to convey the extent of their symptoms in a way that satisfies state requirements. Forcing patients to justify their inability to maintain a consistent work schedule before they can receive or continue to receive care could result in a devastating loss of coverage throughout the rare disease community.

Even more troubling, there are some states that have proposed tying work requirements to lifetime limits on Medicaid coverage. NORD strongly opposes lifetime limits to health care coverage under any circumstance. Lifetime limits disproportionately harm individuals with rare diseases due to the often genetic, life-long nature of their disease, as well as the incredibly expensive therapies and orphan drugs used to treat them. Rare disease patients who are subjected to work requirements would suddenly find themselves entirely without coverage, regardless of whether they have adhered to the work requirements.

Finally, states are debating a number of proposals that reduce eligibility and benefits for those with incomes at or around 100 percent of the Federal Poverty Level (approximately $12,140 per year for an individual). Such proposals to weaken access to specialists or other critical services in Medicaid would be exceptionally detrimental to individuals with rare diseases, as continuity of care is essential to effective treatment. Consequently, NORD opposes any proposal that would considerably weaken health care coverage for any population of rare disease patients.

These concerns are not exhaustive, but they are representative of the ways in which the rare disease community might be harmed by some of the emerging proposals to control costs. Medicaid exists to be a safety net for those who cannot access other forms of health care coverage. Substantially altering the program in ways that reduce benefits for people in need is not only diametrically opposed to the purpose of the program, but it will serve to worsen heath care outcomes and increase costs for rare disease patients and their caregivers. As the leading representative of the rare disease patient community, NORD will continue to engage with states on the best way to improve health care through the Medicaid waiver process.

Medicaid

METHODOLOGY

The grading rubric for the entire Medicaid section is included on page 28. States were graded on each of the following categories:

Eligibility for Parents of Dependent Children:
We analyzed at which income level (FPL) states allow parents of dependent children to enroll in Medicaid.

Eligibility for Childless Adults:
States that have not expanded their Medicaid programs do not allow childless adults to enroll in Medicaid, regardless of their income. We looked at whether states have expanded their Medicaid program for childless adults.

Eligibility for Pregnant Women:
All states allow pregnant women to enroll in Medicaid (or through CHIP) but income eligibility can vary widely.

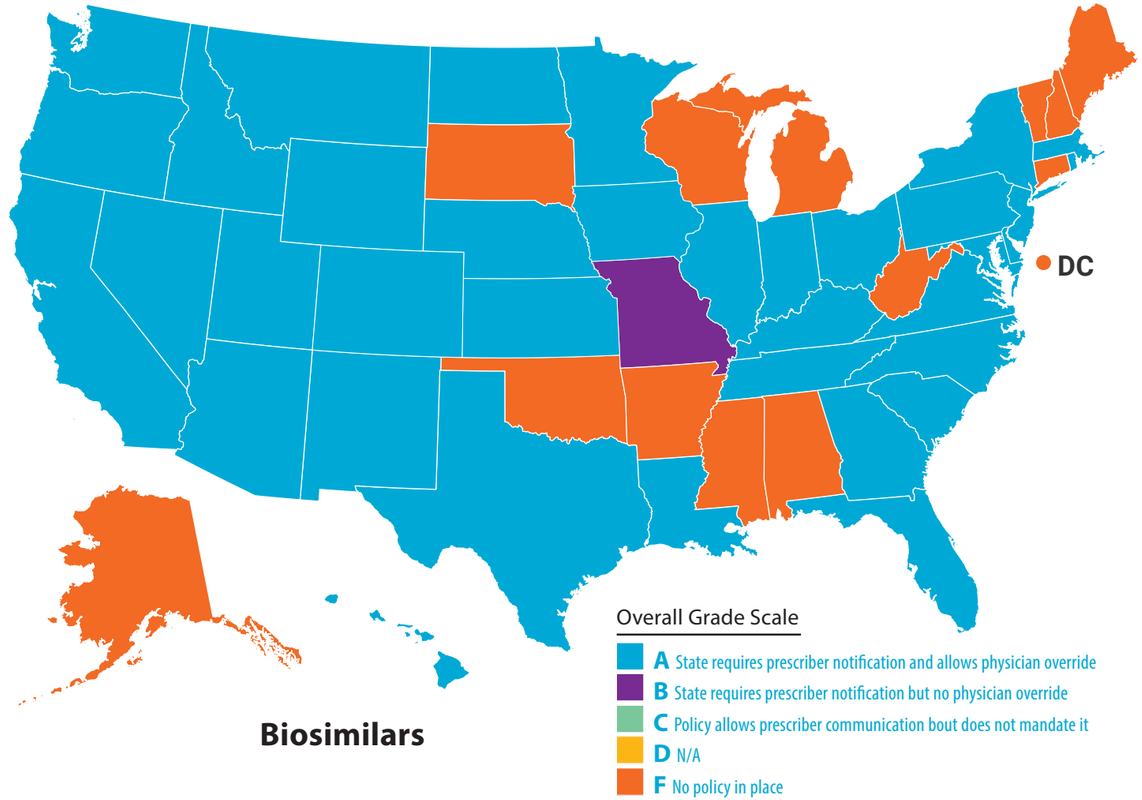
Eligibility for Children (Including CHIP-Funded Eligibility):
All states provide Medicaid (sometime via CHIP) for children ages 0-18, however, financial eligibility for these families can vary widely.

Medicaid/CHIP Eligibility

Table 6: Medicaid Eligibility Grading Rubric

	DESCRIPTION			
GRADE	**Eligibility for Parents of Dependent Children**	**Eligibility for Childless Adults**	**Eligibility for Pregnant Women**	**Eligibility for Children**
A	138% of FPL or greater	138% of FPL or greater	Medicaid/CHIP eligibility of 220% of FPL or greater	Medicaid or CHIP eligibility of 300% of FPL or greater for all age groups (or separate CHIP
B	100%-137% of FPL.	100%-137% of FPL.	Medicaid/CHIP eligibility of 190% to 219% of FPL	Medicaid or CHIP eligibility of 195% to 299% of FPL or greater for all age groups (or Separate CHIP)
C	90% to 99% of FPL	90% to 99% of FPL	Medicaid/CHIP eligibility of 150% to 189% of FPL	Medicaid or CHIP eligibility of 150% to 194% of FPL or greater for all age groups
D	89% of the Federal Poverty Level (FPL) or less	89% of the Federal Poverty Level (FPL) or less	Medicaid/CHIP eligibility of 149% of FPL or less	N/A
F	N/A	No coverage	N/A	N/A

Biosimilar Prescriber Communication



BACKGROUND

Biologics represent the future of rare disease treatments. Harvested from living organisms, biologics treat rare and chronic diseases in an innovative and rejuvenating manner that small molecule treatments are unable to do. Biologics are especially promising, but they also require increased research and development time due to their extremely complex nature.

As new biologic treatments have been developed, we have also seen increased development of so-called "biosimilars," which are treatments that are derived from original biologic that will soon come off patent. There is a tendency to think about biosimilars similarly to how we think about generic drugs – i.e., a molecularly entity identical to the original drug. However, due to the sensitive manufacturing process of biological products, even the slightest change can have a significant negative impact on a patient's therapeutic regimen. This is a serious issue for a large segment of the rare disease community because not all drugs work the same for every patient, especially when dealing with unpredictable disease progression.

This has profound implications for how biologics are

Biosimilar Prescriber Communication

prescribed and dispensed. As more biolsimilars are developed, there is a tendency in states to use them as lower cost substitutes for patients without considering the specific molecular differences. To ensure patient safety and promote access to biosimilars, health care providers need to know which medicine was dispensed to the patient, whether a substitution was made, and if so, to what alternative product. These factors are all critical pieces of information that need to be taken into consideration when supplying a patient with medication.

In light of this challenge, prescriber communication between a pharmacist and a doctor about which biological product has been dispensed can help address this important concern to the rare disease community. As of February 2018, 46 states have passed laws requiring communication with a prescriber before a biosimilar can be dispensed. Many of these states also provide a straightforward process for the prescribing physician to overrule the dispensing of a biosimilar based on medical needs.

NORD strongly supports state legislation that ensures pharmacists will be required to communicate – to a patient's prescribing physician – the dispensing of a substitute biological product for another biologic drug.

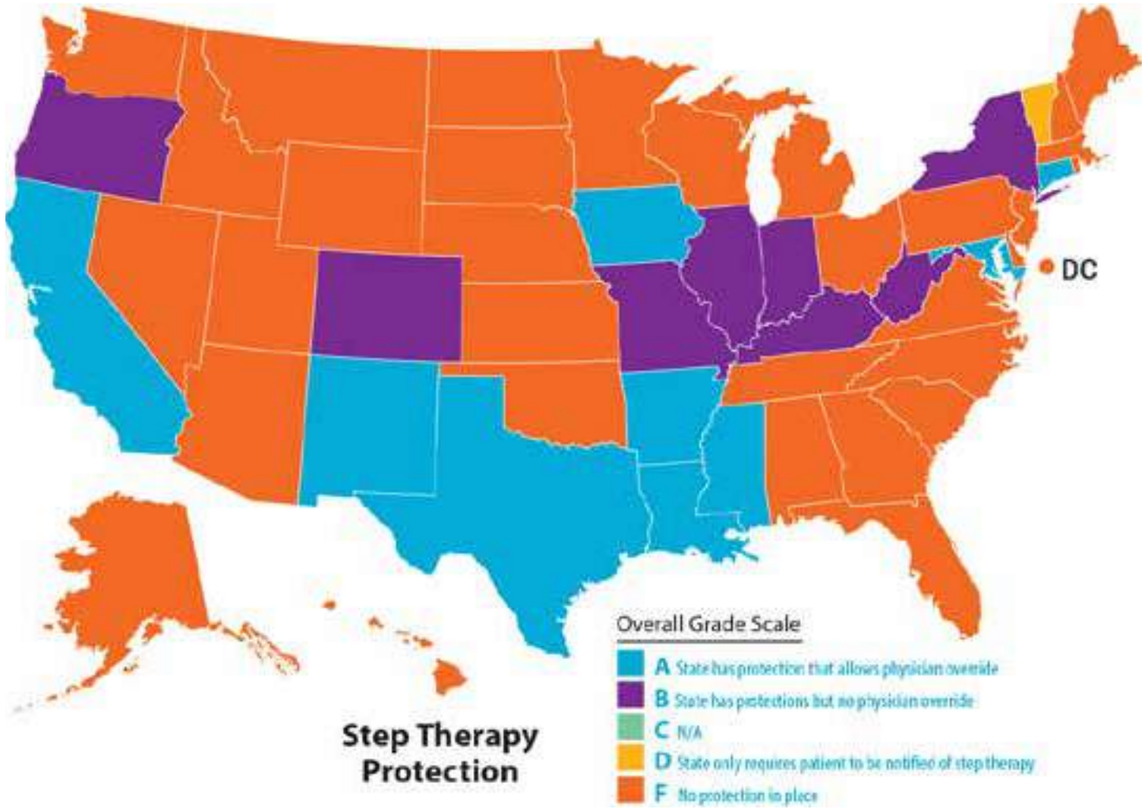
Table 7: Biosimilars Grading Rubric

GRADE	DESCRIPTION Biosimilars Grading Rubric
A	Policy requires pharmacist to notify prescriber before making a substitution. Policy allows physician to override biosimilar substitution
B	Policy requires communication but does not allow for physician override
C	Policy includes prescriber communication but does not mandate it
D	--
F	No policy

METHODOLOGY

In analyzing state policy pertaining to biosimilar prescriber communication, NORD focused on state polices that both require communication between the pharmacist and prescriber and allow for the physician to override a substitution. State policy with both of these features earned an A. States that required communication but did not provide an override earned a B. The full grading rubric for this section can be found below. Due to the nature of this policy, there were no circumstances that warranted a D grade.

Patient Protections for Step Therapy



BACKGROUND

Step therapy (a.k.a. fail first) is a procedure by which insurers (public or private) require a patient to take one or more alternative medications before being put on the medicine preferred by their provider. While this is done by insurers as an attempt to control health care costs, step therapy has been increasingly applied to patients with little regard to their medical situation or treatment history. As a result, in many cases step requirements can delay appropriate treatment and ultimately increase costs, not lower them.

As the use of step therapy has increased (at least 60 percent of commercial health plans have implemented it[1]), so has the need for states to ensure that these requirements do not needlessly interfere with appropriate care for patients. For instance, in some cases, patients switching insurance plans may be required to take go off a successful treatment and take a less effective medicine simply because it is also less expensive.

NORD supports state efforts to place adequate patient protections around the use of step therapy that will ensure

Patient Protections for Step Therapy

patients are protected. The main features of these protections are as follows:

1. Ensure step therapy is based on medical criteria and clinical guidelines developed by independent experts
2. Create a simple and accessible exceptions process for providers and patients to challenge the use of step therapy
3. Establish a basic framework for when it is most appropriate to exempt patients from step therapy

These protections will protect patients while still enabling health plans to achieve the cost-saving benefits of step therapy when it is appropriate.

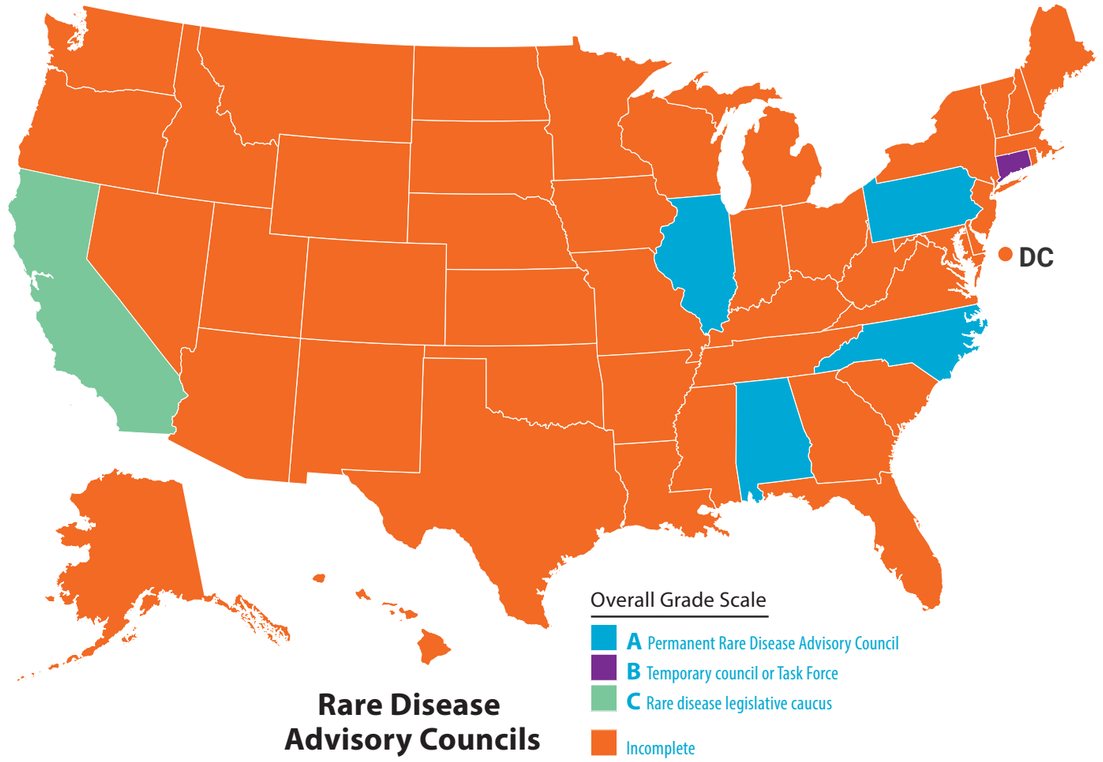
METHODOLOGY

NORD graded states on the three criteria it believes are most important to protecting patients against the inappropriate use of step therapy. States with policies that meet all three requirements (1: policies based on independent medical criteria; 2: clear exceptions process; and 3: allowing physician override) earned an A grade. States that do not allow for physician override earned a B, and states that only require for patients to be informed earned a D grade. The full rubric can be found below. For the purpose of this category, there were no conditions in which a state could earn a C grade.

Table 8: Step Therapy Grading Rubric

GRADE	DESCRIPTION
	Step Therapy Grading
A	State requires step therapy to be based on independent medical criteria. State requires plans to establish a clear exceptions process for patients and providers. State allows prescribing physician to override step-therapy based on medical criteria.
B	State requires step therapy to be based on independent medical criteria. State requires plans to establish a clear exceptions process for patients and providers.
C	--
D	State only requires patients to be notified of the implementation of step therapy.
F	No policy

State Rare Disease Advisory Councils



BACKGROUND

The 2018 State Report Card goes into detail on the concrete policy changes states can make to ensure better access to care for rare disease patients. However, it is often the case that addressing these needs begins with simply ensuring that the rare community has a voice in government. Several states have recognized this and worked with local advocates to create new Rare Disease Advisory Councils (aka a Task Force or Commission).

The purpose of these councils is to evaluate and make recommendations to the state on issues related to health care access and coverage for rare disease patients as well as disseminate information on specific rare diseases. Further, by mandating broad participation among different government agencies on their rare disease council, these states have helped ensure greater awareness and education on rare disease among state leaders and decisions makers.

Ultimately, NORD believes that the establishment of a focused rare disease advisory council can help pave the way for better health care policy in a given state, therefore, we are strongly supporting the work of local advocates to create new councils in their state.

State Rare Disease Advisory Councils

To date, states with existing Rare Disease Advisory Councils (such as Pennsylvania and North Carolina) have shown that this model is an effective way to increase government awareness on issues like newborn screening and Medicaid restrictions for new orphan drug approvals.

METHODOLOGY

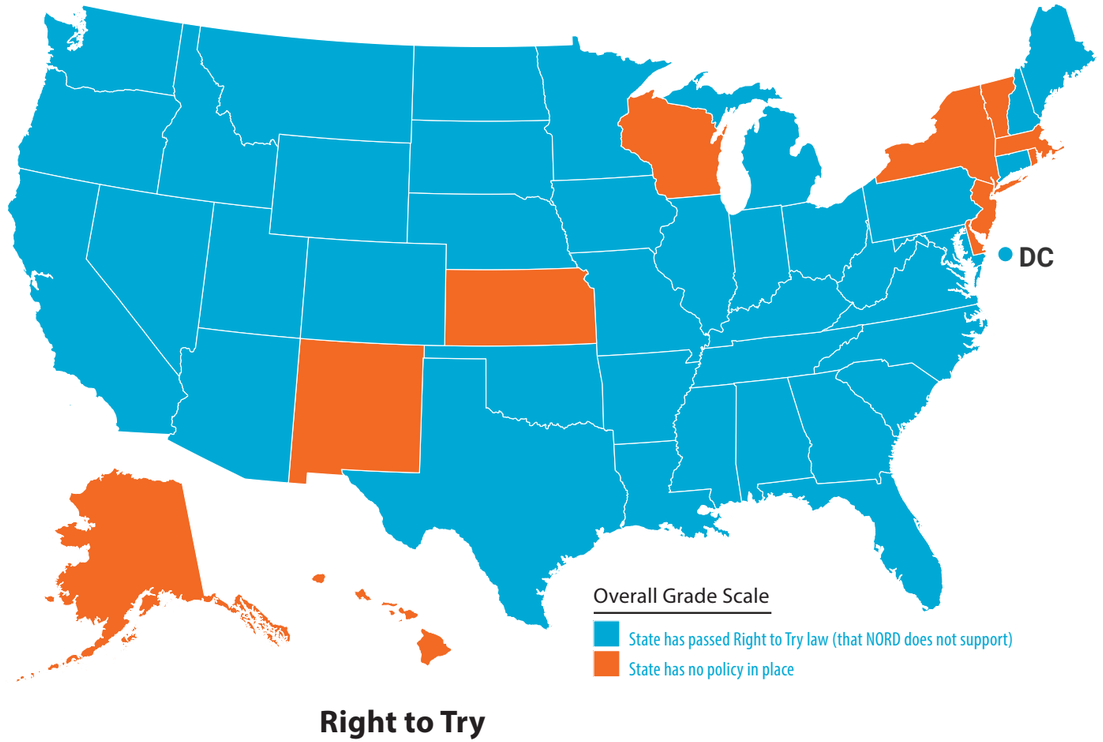
Given the relatively recent onset of this policy issue, along with the lack of state awareness about rare disease councils, NORD only graded states that have enacted a new policy. For states that have not implemented an advisory council, grades are marked as incomplete. In addition, many states have existing advisory structures that are not specific to rare diseases but may serve this function. In these states, it may not necessarily be appropriate to create a new advisory council.

The grading rubric on the next page details how NORD evaluated current and proposed advisory councils. The complete analysis is available in the appendix to this report.

Table 9: Rare Disease Advisory Councils Grading Rubric

	DESCRIPTION
GRADE	**Rare Disease Advisory Councils Grading**
A	Permanent Rare Disease Advisory Council
B	State established temporary Rare Disease Task Force
C	Rare Disease Legislative Caucus
D	No formal body to address rare disease policy issues
F	No formal body to address rare disease policy issues

Right to Try



Right to Try

BACKGROUND

NORD strongly supports patient access to unapproved therapies. However, the Right to Try bills that have been passed in states do not enact policy changes that would give rare disease patients greater access to promising investigational therapies. Instead, these bills are likely to do more harm than good.

First of all, the Food and Drug Administration (FDA) already allows access to experimental therapies through expanded access programs. All Right to Try proposes to do is remove FDA from the initial approval process for accessing an investigational therapy outside of a clinical trial. Removing FDA from this process is not likely to facilitate increased access to investigational therapies because FDA currently approves 99.7 percent of all expanded access requests submitted by physicians and companies for patients with immediately life-threatening illnesses who cannot participate in clinical trials.[1] Further, the Government Accountability Office (GAO) has found that substantial changes were not needed within the FDA program, aside from greater clarity on the use of adverse event data.[2]

In reality, it is almost always the company that prevents access

Right to Try

to experimental treatments because of concerns about safety and the impact on ongoing clinical trials. Right to Try does nothing to address the reasons why companies refuse access to their experimental treatments.

Second, Right to Try definitions of who qualifies for access is broad enough that it could shift health policy precedent and undermine patient safety standards. For example, in many states, Right to Try does not require that an experimental drug be subject to ongoing clinical trials. This means that patients could be unknowingly taking treatment that has failed to demonstrate any effectiveness or has substantial safety risks.

Further, most state Right to Try bills do not require that patients first seek enrollment in a clinical trial or that they be educated about other programs that could help them (such as expanded access). This not only undermines the drug development process but is a disservice to patients who may not be informed about all of their options for accessing investigational therapies.

NORD is not alone in its concerns about the impact of Right to Try:

Dr. Scott Gottlieb, FDA Commissioner:
"I think there is a perception, ...that there are certain companies and products that aren't necessarily being offered under the current construct and the Right to Try legislation might provide more of an incentive and an opportunity. ... I don't necessarily see that same opportunity because I think the biggest obstacle to offering drugs through expanded access is the supply constraints." [3]

Mr. Kenneth Moch, President and CEO of Cognition Therapeutics:
"...the argument that Right to Try legislation is going to make more people have access to experimental medicines does not exist in my mind as a drug developer nor in anybody I know, and I can't say it more bluntly than that."

Right to Try legislation is going to make more people have access to experimental medicines does not exist in my mind as a drug developer nor in anybody I know, and I can't say it more bluntly than that."

Dr. Ellen Sigal, Chair of Friends of Cancer Research:
"Any legislation that goes forward cannot circumvent the FDA and must be carefully crafted to assure that we do not create a loophole for those seeking to profit off the sick by offering false hope... we must not subject patients to false hope or unacceptable side effects." [4]

Ultimately, NORD believes it is important to remember that the current regulatory system for medical products and research in the United States was created as a result of serious patient harm and exploitation that occurred early in the 20th Century. Birth defects resulting from Thalidomide are an example of what happens when drugs are given to humans without proper safety review and approval. While obtaining unapproved therapies outside of a clinical trial is not about research, the products themselves remain experimental and have not been shown to be safe and effective. Clinical research subject protections are in place when experimental products are being tested to ensure the safe and ethical treatment of research participants. Patients seeking expanded access to unapproved therapies outside of clinical trials must be afforded the same ethical standards and protections as patients taking part in clinical trials.

LOOKING FORWARD

As NORD continues to improve and expand our work at the state level, there are several policies we are engaged on that are not included in this iteration of the ***State Report Card.*** These are issues that NORD has identified as priorities for state action and may be included in future editions of the Report Card.

EMS PROTOCOLS FOR RARE DISEASES

One of the immense challenges facing many rare disease families is how to ensure proper treatment and care in emergency situations. Whether at the emergency room or with a paramedic, many emergency medical services (EMS) personnel do not have the resources to ensure rare disease patients are properly treated. In analyzing ways to improve EMS care for rare disease patients, NORD has identified the following issues:

- Legal restrictions preventing EMS personnel from giving self-administered medication to patients.
- Lack of ER access to rare disease information treatment protocols that prevent unnecessary or inappropriate treatment.
- Lack of education and training on rare disease for providers, public safety personnel, and schools.
- Unexpected medical bills resulting from out-of-network emergency care.

NORD is currently exploring different policy options to address these challenges and may include state action on EMS care in the next Report Card.

TELEMEDICINE

Telemedicine offers an opportunity that is particularly beneficial to the rare disease community: better access to medical specialists who may have unique knowledge and experience treating rare diseases. In addition, telemedicine can help reduce disparities in access to care for all types of medical needs and ultimately increase health care quality. However, there are several roadblocks that may prevent rare disease patients from experiencing the benefits of telemedicine, such as restrictions in physician licensure, prescribing authority, parity in reimbursement, and coordination with local providers.

NORD is currently developing our position on many of these issues and determining how states can implement telemedicine in a way that ensures access for the rare disease community.

PRECISION MEDICINE AND GENETIC TESTING

Next generation genetic sequencing represents one of the most promising methods for diagnosing individuals with rare, genetic disorders. For many, if not most, rare diseases, genetic sequencing is the only reliable method for diagnosis. The advent of new technologies has brought hope to millions of Americans with rare diseases still waiting for an accurate diagnosis. On average, individuals with a rare disease wait seven to ten years to obtain an accurate diagnosis, leaving many individuals with chronic conditions still waiting for a diagnosis. There are millions of patients in the U.S. who are still undiagnosed, and genetic testing may be their only hope.

NORD supports increasing coverage to appropriate forms of genetic testing in order to address medical needs (such as the lack of a clinical diagnosis). We also recognize that the coverage and availability of testing is a barrier to the expansion of genetically-targeted medicines. These advances, broadly referred to as "precision medicine," have the potential to lower costs and produce better health outcomes by eliminating waste, avoiding unnecessary treatment, and reducing the incidence of chronic disease. However, without improvements in how states mandate coverage of genetic testing and reimburse for genetically-targeted therapies, rare disease patients are less likely to benefit from these advances.

PRIVATE INSURANCE MARKET STABILIZATION

Over the past year, the Federal government has taken various actions to destabilize private insurance markets across the country.

Prior to the 2018 open enrollment period in the Affordable Care Act (ACA) marketplaces, the Trump Administration cut the open enrollment period in half, and substantially reduced

LOOKING FORWARD

the resources for certified health insurance navigators and enrollment assistants. In November, the Administration proposed various changes to the marketplaces within their Notice of Benefit and Payment Parameters Proposed Rule that could allow states to weaken their essential health benefit and network adequacy requirements. In December, Congress repealed the ACA individual mandate as part of the Tax Cuts and Jobs Act. The Trump Administration is also proposing to expand the use of association health plans and short-term plans, both of which include more relaxed requirements for coverage and benefits.

Each of these actions by the Federal Government effectively destabilize private insurance markets within states. There are, however, various actions states can pursue to counter or mitigate these destabilizing actions. In our next ***State Report Card,*** we will evaluate states on their efforts to stabilize and strengthen their health insurance markets and ensure individuals with rare diseases and their families maintain adequate and affordable coverage.

For example, states can enact or implement the following policies to strengthen, or at least maintain, their private health insurance markets:

Create a state-level individual mandate: With the repeal of the federal individual mandate, healthy individuals no longer have a financial incentive to purchase health insurance. This could raise premiums for those with rare diseases left within the marketplaces and potentially price our patients out of the marketplace. States can avoid this situation by enacting their own individual mandate, or similar inducement, to ensure healthy individuals participate within the marketplace.

Obtain a 1332 reinsurance waiver: States have successfully stabilized premiums by requesting and receiving a 1332 waiver from the federal government to create a reinsurance program for particularly expensive beneficiaries. This has allowed our patients to remain covered while premiums remain stable for the entire marketplace. States should seek these waivers from the Federal government to create these programs.

Enact additional marketplace consumer protections: With the potential for short-term plans and association health plans to further segment the marketplaces between the sick and healthy, states can enact policies to prevent such segmentation by requiring all potential plans to comply with critical consumer protections. These include EHB and network adequacy requirements, as well as community rating requirements, benefit exclusion disallowances, and guaranteed issue.

Create state essential health benefit requirements: As the Federal government continues to undermine the ACA essential health benefits, states can enact their own EHB packages to ensure rare disease patients are offered quality coverage

Invest in Enrollment and Coverage Efforts: With the Federal government reducing resources for outreach for health insurance enrollment efforts, states can conduct their own outreach efforts to ensure consumers are aware of the opportunity and signing up for health insurance.

Resources

RARE ACTION NETWORK™

The ***State Report Card*** is one of a number of tools available through NORD's Rare Action Network. The mission of the Rare Action Network (RAN) is to connect and empower a unified network of individuals and organizations with tools, training, and resources to become effective advocates for rare diseases through national and state-based initiatives across the United States.

RAN is the nation's leading rare disease advocacy network working to improve the lives of the 30 million Americans impacted by rare disease. The goal of RAN is to ensure that the rare disease community is represented and supported in all 50 states.

RAN serves as a broad spectrum of stakeholders ranging from patients, to their families, caregivers, and friends; from researchers to industry; to physicians and academia. While working on both the national and state level, RAN filters information to help address issues of national concern and engage rare communities to take action through policy, awareness, and education initiatives in their state and local communities.

JOIN THE NETWORK

Members of the Rare Action Network are part of 30+ million-person community working towards improving the lives of patients with rare diseases. This expansive Network enables you to:

- Connect with other patients, caregivers, and stakeholders, within your state and region through calls, webinars, and in-person meetings;
- Participate in regional and local events to connect, learn, and address the nation's leading issues;
- Develop relationships with key decision-makers and opinion leaders;
- Share your story to help raise awareness;
- Receive news and information on what the Network is taking action on and participate in the Network's calls to action.

To learn more about NORD's advocacy and sign up for the Rare Action Network, please visit www.rareaction.org

HELP US BUILD THE NEXT ITERATION OF THE STATE POLICY REPORT CARD

The development of this report is an ongoing process for NORD. For future iterations of the report, we plan to incorporate the following issues in some form:

- Coverage and reimbursement of genetic testing
- Coverage of medical foods for allergic disorders
- State policies concerning false allegations of medical child abuse pertaining to children with a rare disease
- State policies governing Institutional Review Boards (IRBs) for clinical trials

Have an idea or input of what we should look at? Let us know! We welcome the feedback and the opportunity to work with you. Send us a note at orphan@rarediseases.org. You can also reach on Twitter (@rareaction and @rarediseases) and Facebook (facebook.com/NationalOrganizationforRareDisorders)

Sources by Topic

Medical Food Sources

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Connecticut

55 Kenosia Avenue
Danbury, CT 06810
203.744.0100

Washington, D.C.

1779 Massachusetts Avenue, NW
Washington, D.C. 20036
202.588.5700

Massachusetts

1900 Crown Colony Drive
Quincy, MA 02169
617.249.7300

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