2016

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 the 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 500 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, and 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 support and 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 and 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. Due to partisan disagreements, efforts to refine the ACA to address these shortcomings were unsuccessful.

With patient access to health care becoming increasingly dependent upon state policies, in 2015 NORD launched the first-ever report to evaluate how states are serving people with rare diseases. We are pleased to present the second annual edition to demonstrate where progress has been made and where it is still needed. Looking to the future, the results of the recent U.S. election have posed new challenges for NORD and the rare disease community, and we are preparing to work with the new Administration and the new 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 and our elected officials at the federal and state levels to protect rare disease access to affordable treatment and care, and to continue to make advancements. 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

In 2015, the launch of NORD's inaugural State Policy Report altered the landscape for rare disease advocates by empowering them to analyze and advocate for health care policy decisions determined at the state level that impact their daily lives. We dubbed that report a "Road Map for State Improvement"which rightfully implied that the journey was just beginning, both for NORD and the rare community.

The 2015 report called attention to the lack of policies to ensure access to care for rare disease patients in many states. The policies we focused on in the report were: prescription drug co-insurance, medical foods access, newborn screening, and Medicaid eligibility.

The 2016 State Report Card: A Roadmap for Improvement to Help People with Rare Diseases provides an update on each of these policy categories (spoiler: the news is not great). However, it also adds several new issues that we believe are pertinent to the rare disease community. These issues are as follows:

1. Medical Foods Coverage: There are multiple rare disorders which require special nutrition in order to prevent serious disability and allow for normal growth in children and adults. For people with these conditions, medical foods are the only viable treatment option available. Many states have mandated the inclusion of medical foods within health insurance plans sold within their state. However, in the states which do not have medical food mandates, individuals in need of these particular treatments often require assistance in paying for medical food expenses.

2. Prescription Drug Cost Sharing: Health insurance companies use cost sharing for drugs to encourage patients to try lower-cost medications before turning to more expensive ones. However, it is common for insurance companies to charge patients with a severe condition thousands of dollars in cost sharing each month. To assist patients who find themselves in this difficult situation, several states have passed legislation mandating a limit on out-of-pocket costs for certain medications. Given that new treatments can be expensive to develop (it takes an average of 12.5 years and $1.5 billion in 2014 dollars to bring a new drug from preclinical stage through FDA approval), many manufacturers set the cost high to recoup these expenses, especially given the fact that orphan products are intended to treat small patient populations.

3. Newborn Screening: Newborn screening programs are operated almost entirely at the state level, allowing each state to choose which conditions will be screened for at birth. As a result, a new baby may be screened for different diseases depending on which state they live in.

4. Medicaid Eligibility: States have the option of whether or not to expand eligibility for their Medicaid program (publicly-funded health insurance). As a result, someone who is eligible for health care via Medicaid in one state may not be in another.

5. Biosimilar Prescriber Communication (NEW): Biologics are the future of rare disease treatment. These medicines are created from living organisms to treat rare and chronic diseases in ways other cannot. However, not all biologics are the same, which is why it is important for doctors to be alerted whenever a pharmacy or health insurer wants to change the type of biologic dispensed to a patient.

6. Step Therapy Protection (NEW): Step therapy is a process by which insurance companies require a patient to take one or more alternative medicines before being put on the one preferred by their doctor. 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-therapy can delay better treatment and increase costs.

7. State Rare Disease Councils (NEW): Helping the rare disease community starts with ensuring that patients and families have a voice in government. Several states have recognized this and worked with local advocates to create new Rare Disease Advisory Councils. Their purpose is to evaluate and make recommendations to the state on issues related to health care access and coverage for rare disease patients.

Introduction (continued)

HOW TO USE THIS REPORT

As NORD's state advocacy journey continues, it is helpful to think of this 2016 report as a snapshot (or if you prefer, a selfie on the side of the highway). We want to make note of state progress where it has been made, but also continue to chart out the road ahead.

This report is a tool for policymakers and advocates. Use it to learn more about rare disease policies in your state and what needs to be done.

This report is also a starting point for families first learning about rare diseases or with a loved one that was recently diagnosed. It is likely that the policies covered in this report apply to your own health care needs, and therefore can help you understand what the laws are in your state.

Regardless of how you use it, the picture we are showing you will also be more candid than ever, as this year (and for every iteration of this report moving forward) NORD is grading the states. The decision to move into a grade system is intended to provide a more definitive and straightforward analysis of state progress, or lack thereof.

There is more on the grading methodology in the next section, but suffice it to say that gone are the nebulous "Excellent" and "Satisfactory" categories and in their place the time-tested A through F scale.

METHODOLOGY

HOW NORD EVALUATED THE STATES

States were evaluated and graded on the seven policy sections identified earlier. Each section was evaluated independently, i.e., there is no overall grade for a state – only a score for each policy. 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).

The overall grade for each policy section is based on 0% to 100% scale as follows:

- 0% to 59%: **F**
- 60% to 68%: **D**
- 69% to 79%: **C**
- 80% to 89%: **B**
- 90% to 100%: **A**

To determine an overall grade for each policy section, NORD divided each policy into subcategories that were scored using a tiered system. For example, for medical foods, publicly-funded coverage and privately-funded coverage were scored as separate subcategories within the medical foods section. These scores were then combined to calculate the overall grade for a state's medical foods policy.

The specific criteria for each policy subcategory varied depending on the issue being analyzed (each section of the report goes into more detail on the methodology for that particular issue). However, the basic structure of the tiered scoring was the same for all sections:

- **Tier Score of 0**:
  State has no policy provision for the relevant issue
- **Tier Score of 1**:
  State has some policy in place, but it does not meet the standards of higher tiers
- **Tier Score of 6**:
  State policy on the given issue meets minimum standards
- **Tier Score of 8:**
  State policy meets most, but not all desired standards
- **Tier Score of 10:**
  State policy meets all desired standards. A score of 10 is considered model policy that other states should seek to enact.

SECTION I
NATIONAL OVERVIEW

National Overview

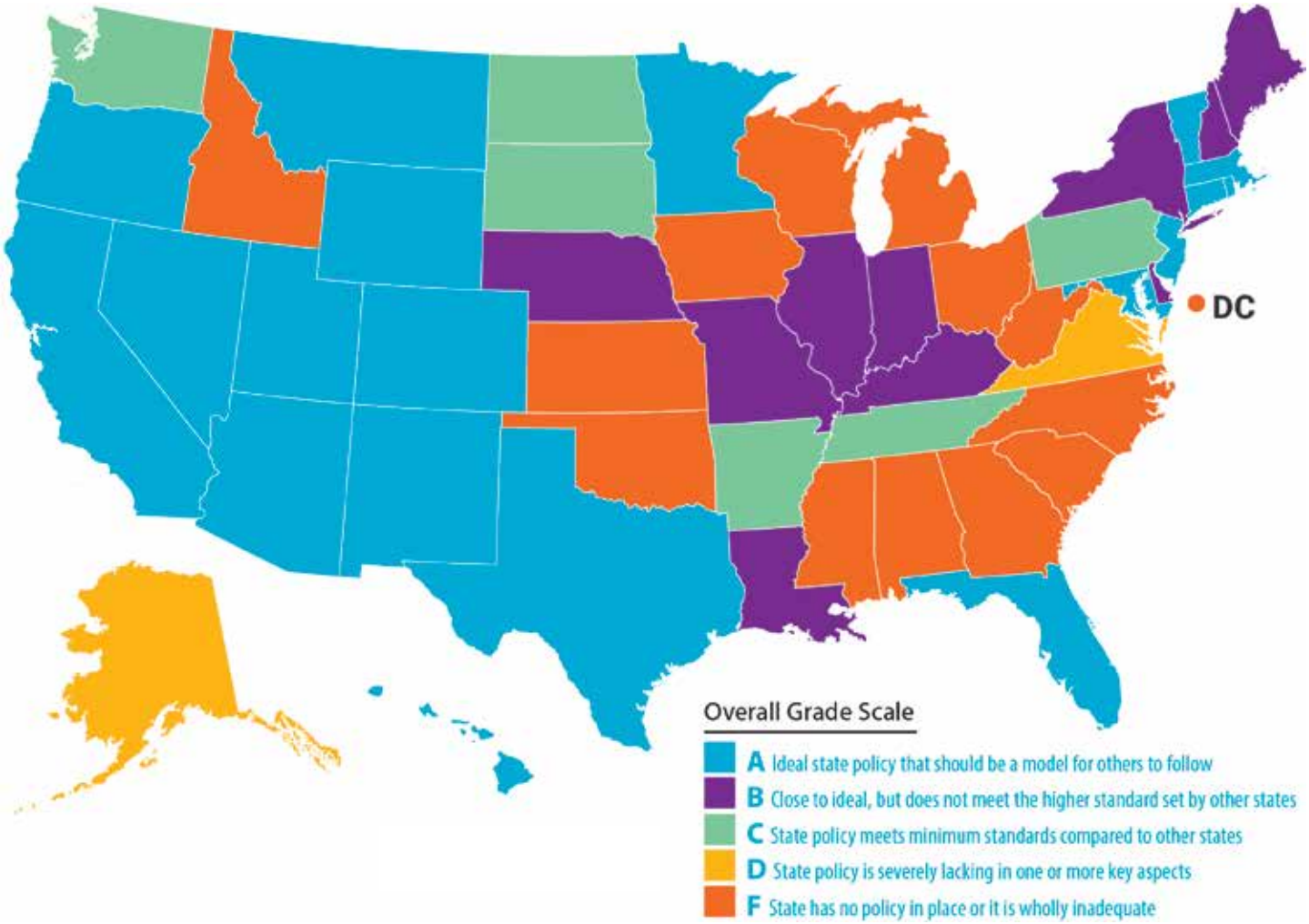
AREAS OF IMPROVEMENT

During 2016, there was minimal improvement from states on the issues NORD analyzed in its 2015 report. For instance, no new state increased their Medicaid eligibility in accordance with the Affordable Care Act. No states passed new laws or regulations protecting patients against high out-of-pocket costs, and only a handful of states added new disorders to their newborn screening program.

However, advocates still had a great impact through their work in each state. For example, new bills were introduced in several new states and more legislators were educated about rare disease. Moving forward, this advocacy work will lay the foundation for more state progress.

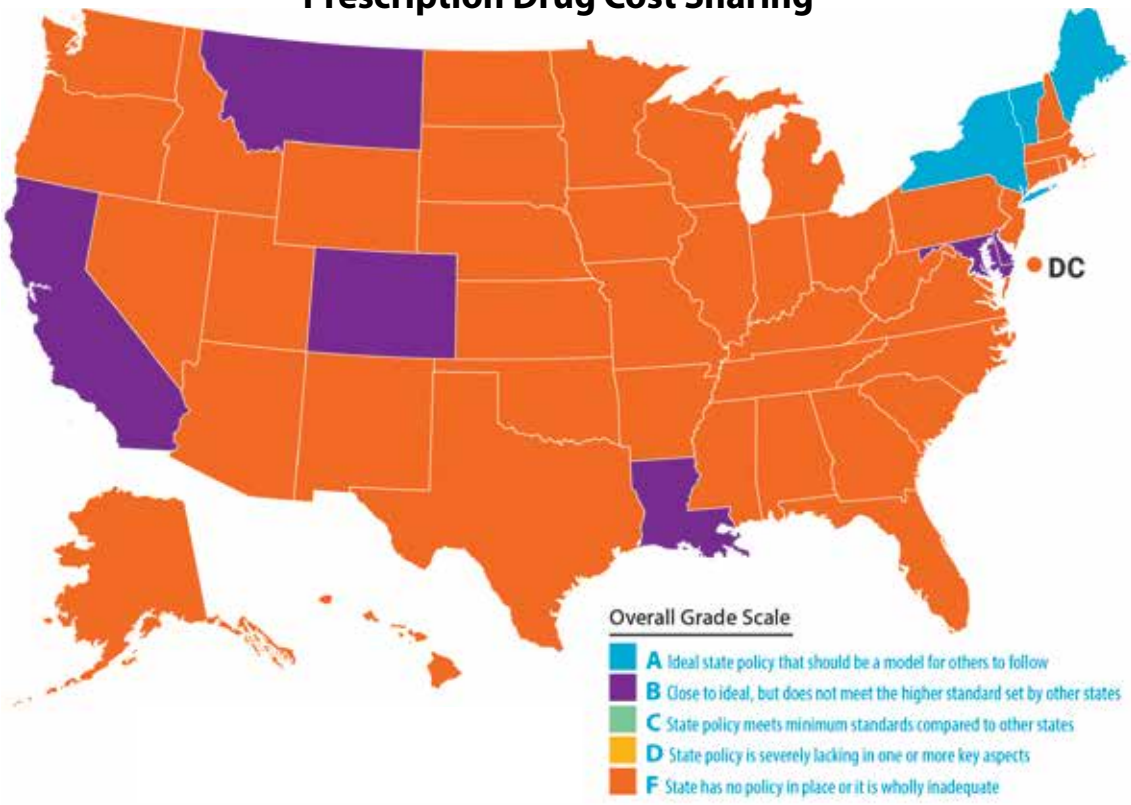
The following maps provide an overview of how each state scored in 2016 across the issues.

Medical Foods

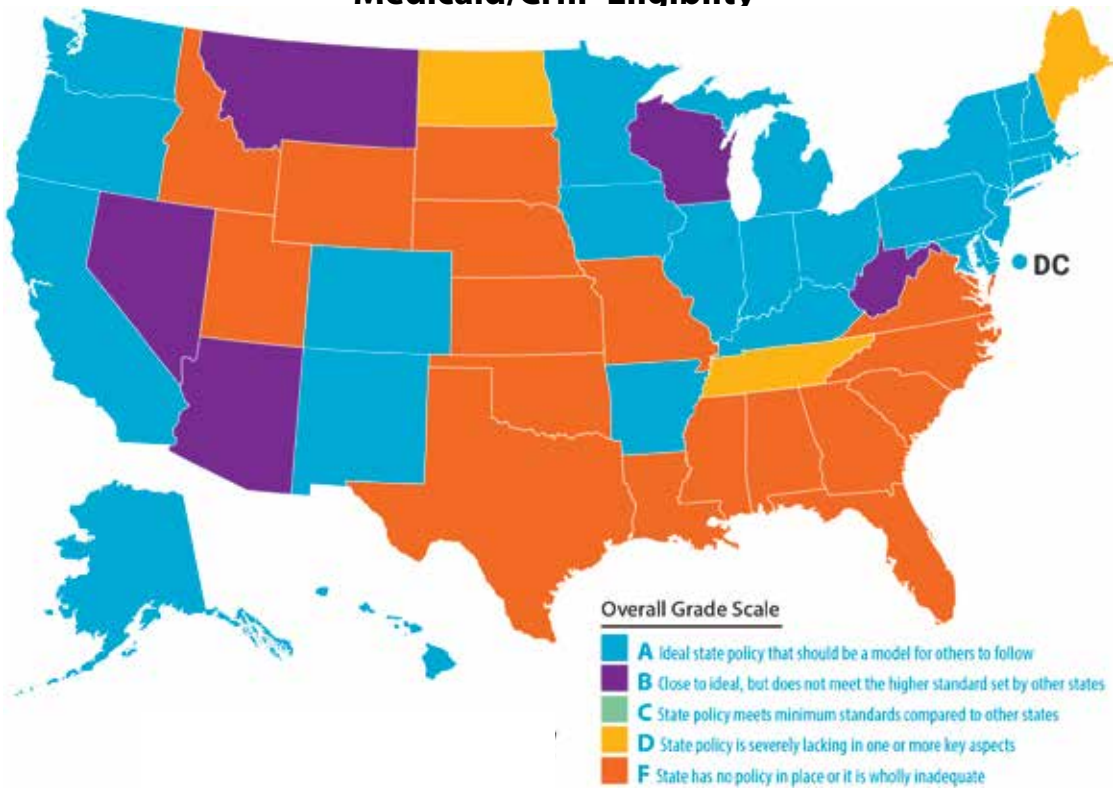


National Overview (continued)

Prescription Drug Cost Sharing

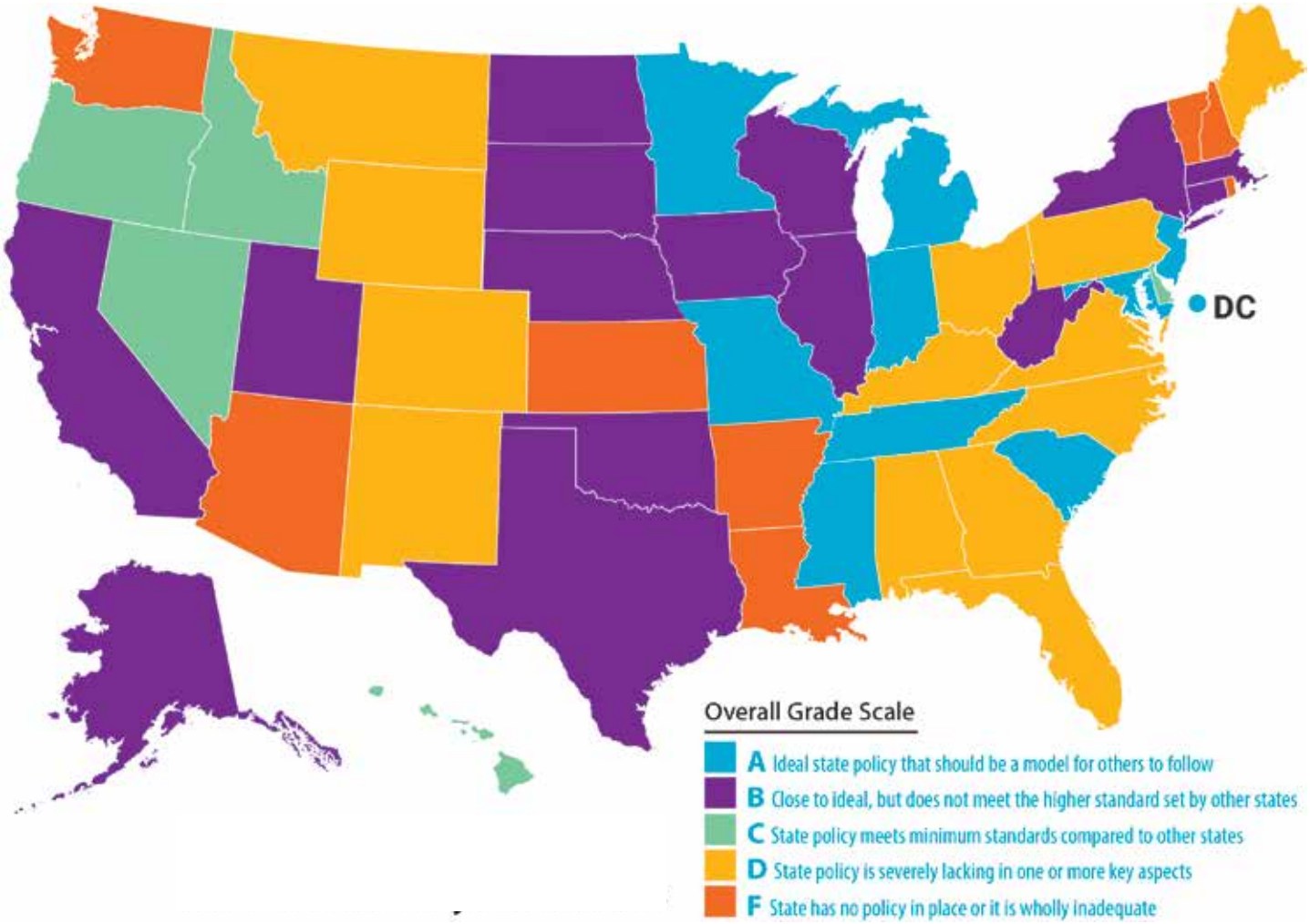


Medicaid/CHIP Eligibilty



National Overview (continued)

Newborn Screening: RUSP Secondary Conditions

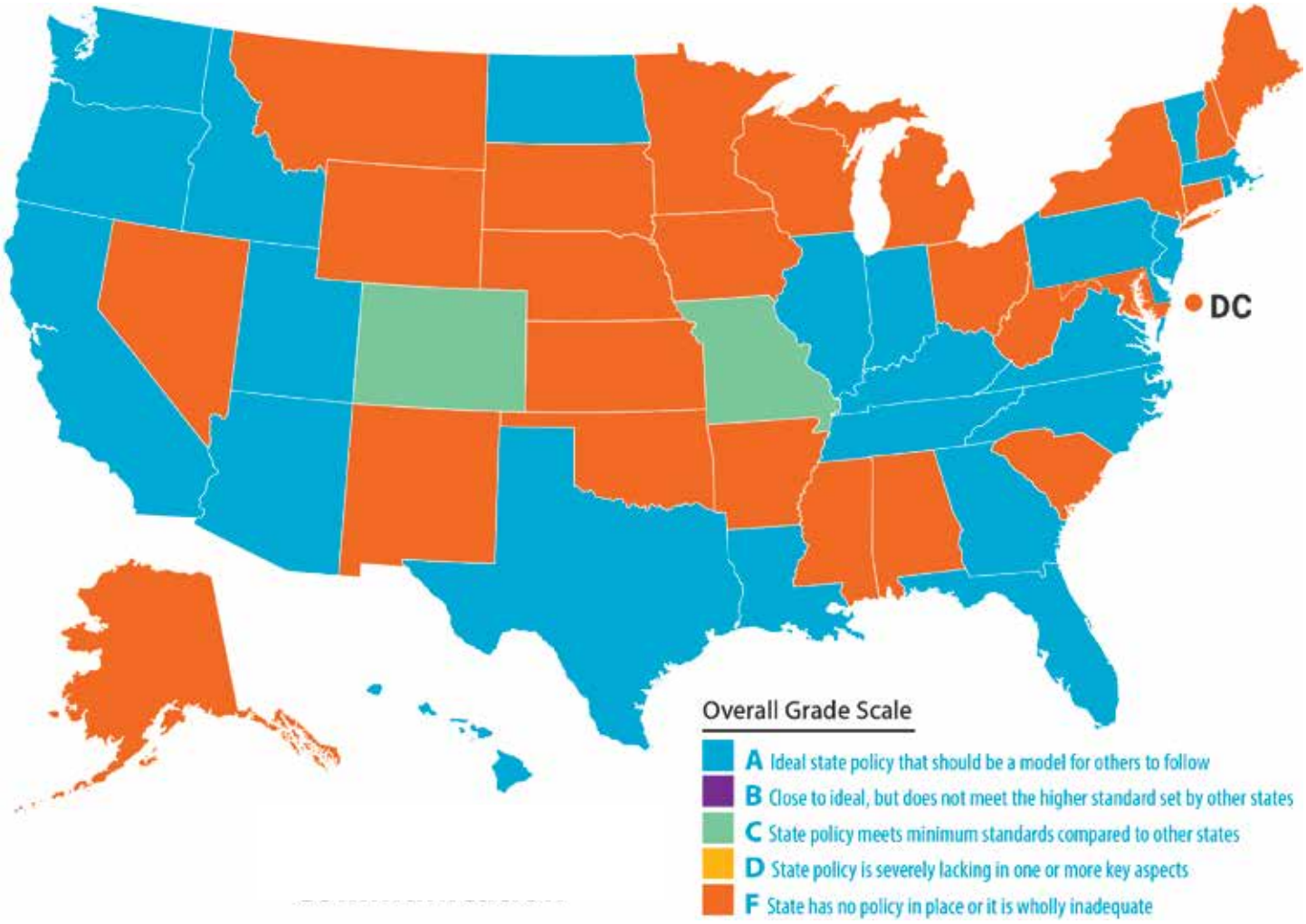


National Overview (continued)

NEWLY EVALUATED CATEGORIES

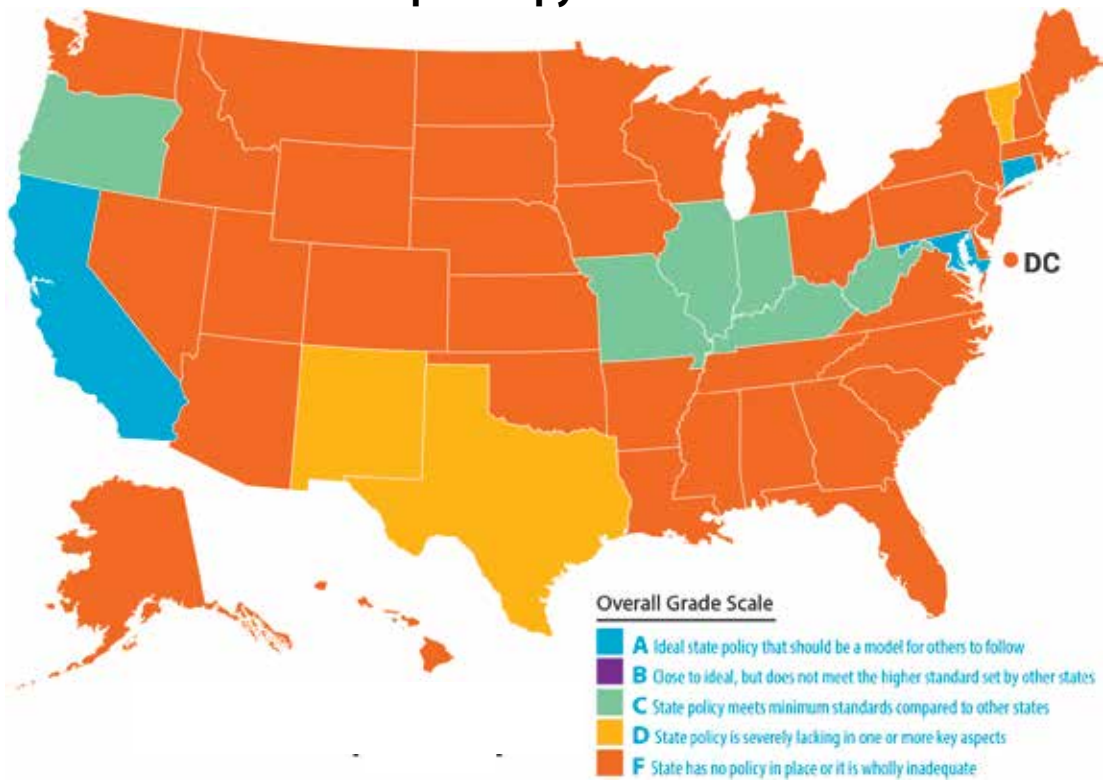
While there has been limited activity on the previous issues NORD has covered in this report, significant legislative progress has been made in the new categories we examined for 2016. Of note, there are now 26 states that have passed laws ensuring adequate communications with physicians when a biosimlar medicine is dispensed by a pharmacy. Moreover, several states have passed new laws ensuring that Step Therapy rules are based on medical evidence and clearly communicated to patients and their doctor. The maps below provide an overview of how each scored in 2016.

Biosimilar Prescriber Communication

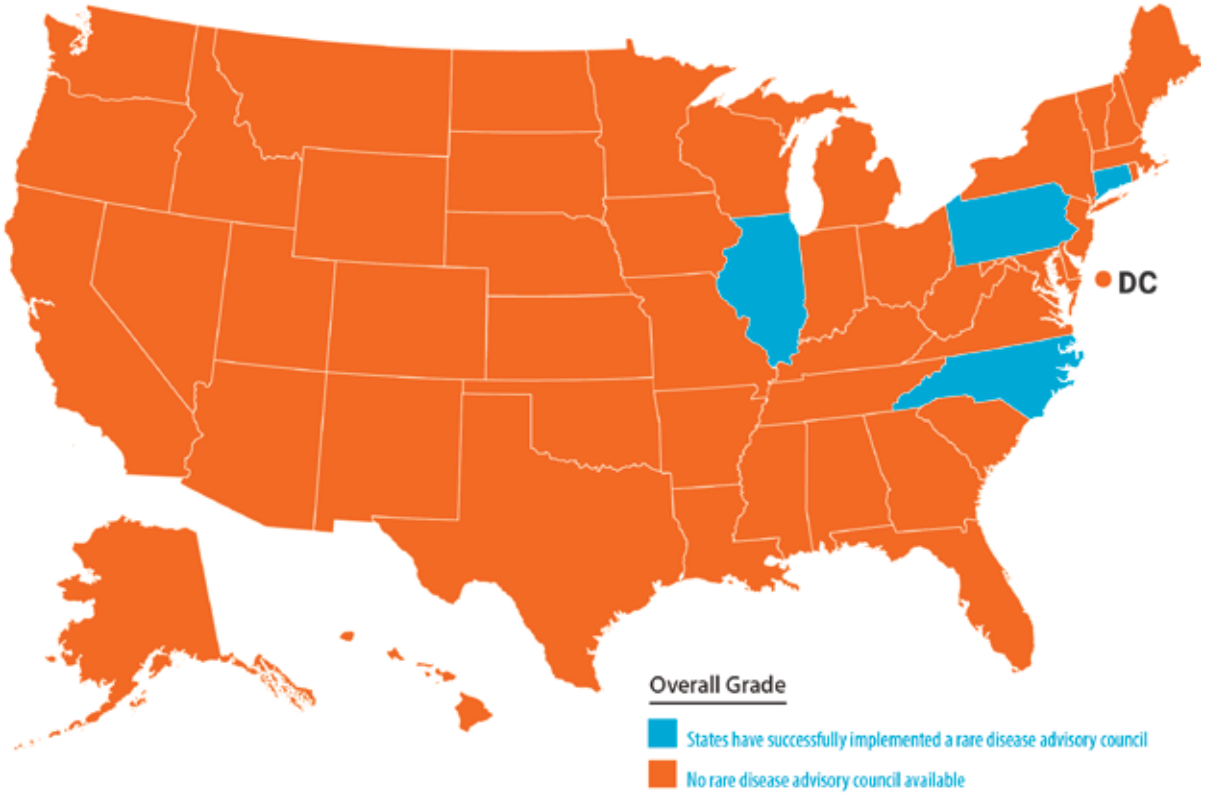


National Overview (continued)

Step Therapy Protection

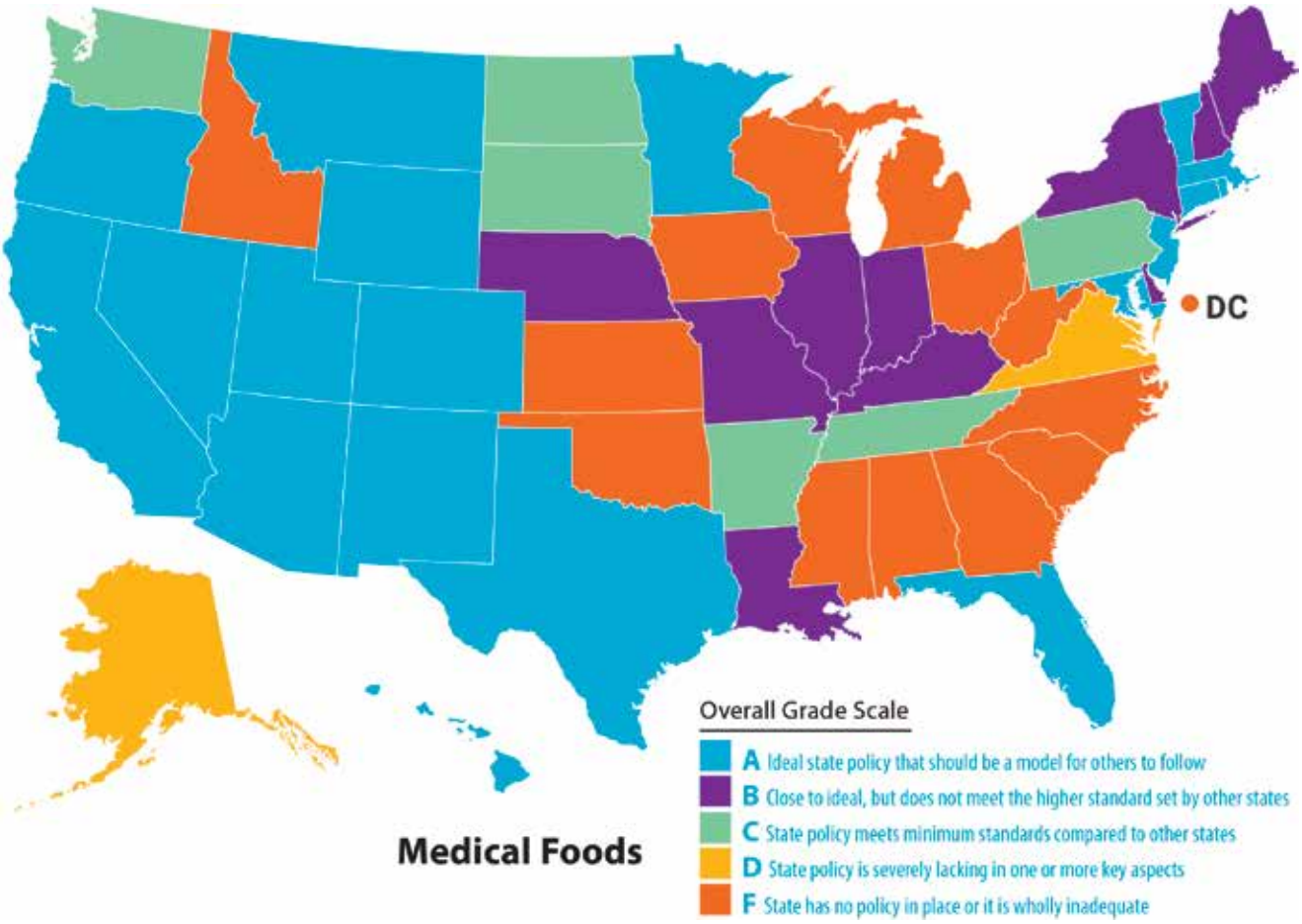


Rare Disease Advisory Councils



SECTION II
EXPLORING THE ISSUES

Medical Foods



Background

There are multiple rare disorders which 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 for inborn errors of metabolism, states also play an integral role in ensuring access to these critical therapies.

Medical Foods (continued)

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 mandates, people in need of these treatments need assistance in paying for them.

Inclusion of medical foods within each state's Medicaid program is also essential, yet only some states mandate coverage. For states that do not provide coverage through Medicaid, a few states have chosen to provide access to medical foods through other public health programs.

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

NORD's Vision

People need special medical nutrition, there is no difference to them between these products and a pill that someone else might take for a different disease. Treatment is treatment. NORD's goal is to ensure that, when supported by the medical evidence, medical foods are covered by insurance the same as any other medical treatment.

29 STATES SCORED AN A OR A B WITH 4 STATES EARNING A PERFECT SCORE CT, CA, MD, OR

Methodology

Table 1 provides the scoring rubric for the evaluation of medical foods policies. States were scored on a tiered system and earned a score of 0, 6, 8, or 10 for each of four separate subcategories.

Scores were assigned to the following four subcategories, for a total possible score of 40 (maximum score of 10 for each category):

- **Covered Diseases for Private Insurance:** This subcategory covers which diseases are eligible for medical foods coverage for private insurance.
- **Mandated Private Insurance Coverage:** This subcategory covers whether or not a state mandates private insurance coverage of medical foods.
- **State-Funded Coverage:** This subcategory covers the level and type of coverage provided by state programs.
- **Covered Disorders for State Programs:** This subcategory covers which diseases are eligible for medical foods coverage for state programs (such as Medicaid).

Results

Overall, 29 scored an A or a B, with four states earning perfect scores (CA, CT, MD, and OR). Most states that earned C's can improve by mandating coverage of low-protein foods instead of just formula-based nutrition. States that earned D's and F's typically do not mandate coverage of any kind for either private insurance or Medicaid.

One area of concern regarding state policy is the process by which certain diseases are covered. In many states, each covered condition is specified in the law, making it difficult to extend coverage for new disorders. NORD believes a better policy is for states to specify coverage based on whether a given disease is part of the newborn screening panel. One obvious drawback of this approach is that it is dependent on state coverage of newborn screening. Currently there are only six states that take this approach.

Medical Foods (continued)

Table 1: Medical Foods Scoring Rubric

	TIER SCORE DESCRIPTION			
SUBCATEGORY	**0**	**6**	**8**	**10**
Covered Disorders for Private Insurance and State Services	State does not mandate coverage of any disorders.	Covered disorders include two or fewer metabolic conditions (such as PKU only).	Covered disorders include three or more metabolic conditions, but not all inborn errors.	All inborn errors of metabolism.
Private Insurance Coverage	State does not mandate private insurance coverage of medical foods.	State mandates private insurance coverage, but sets a limit on eligibility (such as age) or coverage (such as a dollar cap or covering formula only).	State mandates private insurance coverage for both formula and low-protein foods. State has a coverage limit, but it is not less than $2,500 per-person, per-year.	State mandates private insurance coverage for both formula and low-protein foods with no limits on eligibility or coverage.
State-Funded Coverage	State does not mandate coverage for Medicaid. The state does not offer supplemental programs to provide coverage.	State mandates Medicaid coverage for formula, but does not cover low-protein foods; Or State does not mandate Medicaid coverage but provides coverage on a case by case basis.	State mandates Medicaid coverage for formula and low-protein foods, but coverage includes age restrictions; Or State provides formula and low-protein foods through a supplemental program that may have age restrictions.	State mandates Medicaid coverage for medical foods with no age restrictions. Or State provides formula and low-protein foods through a supplemental program with no age restrictions

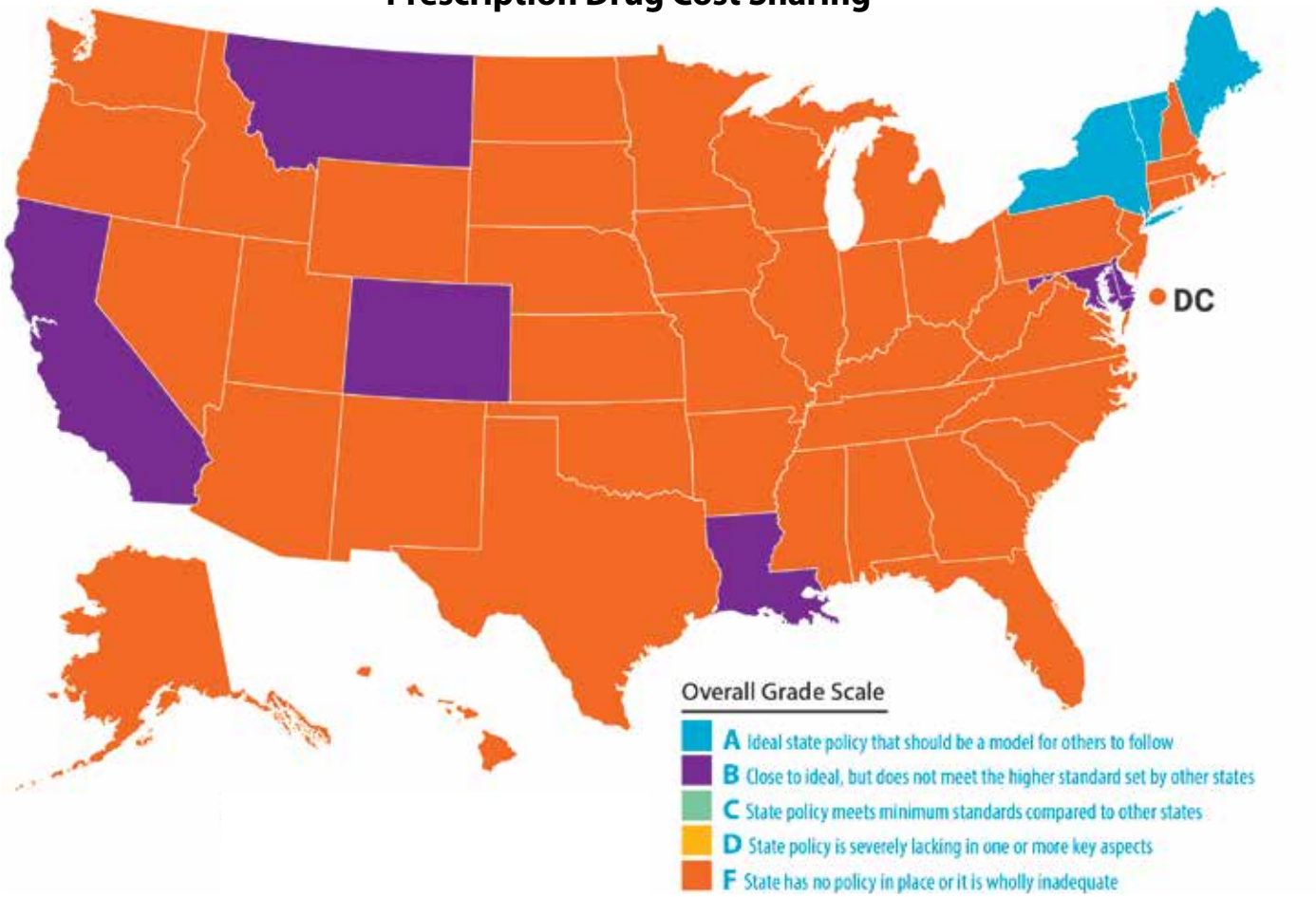
Progress Since 2015

According to NORD's analysis, no state passed new laws or regulations increasing access to medical foods. In fact, only a handful of states even considered such legislation for specific disorders. Part of the reason for this is that the hard data in support of when and which medical nutrition is medically necessary is largely lacking. It has become difficult for both insurance and patient advocacy organizations to definitively assert when a certain type of medical nutrition should be covered and for which disorders. In 2017, NORD will do more work to address these gaps in knowledge to improve coverage for medical foods.

IN 2016 29 STATES NO NEW STATES PASSED LAWS FOR MEDICAL FOODS

Prescription Drug Cost Sharing

Prescription Drug Cost Sharing



Background

Under the Affordable Care Act (ACA), many people with rare or chronic diseases who previously have been denied insurance coverage on the basis of a pre-existing condition are now able to purchase commercial plans. Insurance coverage means these individuals now have reliable access to medications that can help treat and manage their condition. Unfortunately, some insurance doctors have implemented policies that place orphan therapies for rare diseases in a higher cost category associated with specialized drugs, on the so-called "specialty-tier" of a drug formulary. For drugs placed on this tier, 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.[2] Such requirements effectively defeat the purpose of having insurance in the first place.

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.

Prescription Drug Cost Sharing (continued)

The utilization of specialty tiers in these plans is staggering. For example, it is estimated that up to one-fifth of Exchange plans require cost sharing of 30% or higher for the entire class of drugs for common chronic diseases.[3]

To assist patients who find themselves in this difficult situation, several states have passed legislation mandating a 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 insurance 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 cost for all beneficiaries.[4]

NORD's Vision

Despite its original intent, the use of more expensive cost sharing tiers for different diseases has become discriminatory. NORD is working to ensure that regardless of which state someone lives in and what medical condition they may have, their health insurance company cannot deny them treatment by charging them so much in cost sharing that their care becomes unaffordable.

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. One state is considering a policy by which prescription drug costs cannot exceed 20% of a plan's out of pocket limit for all medical costs.

States were scored on a tiered rubric with possible scores of 0, 1, 6, 8, or 10 (see Table 2). Bills that have not passed both legislative chambers were not factored as part of a state's score (as of the release of this report no bill introduced this session has passed).

Table 2: Prescription Drug Cost Sharing Scoring Rubric

TIER SCORE DESCRIPTION				
0	**1**	**6**	**8**	**10**
State does not have a cap on cost sharing.	State has enacted cost sharing limits for a limited number of treatments (such as oral chemotherapy only).	State has instituted a per-drug cap or total cap on cost sharing that does not apply pre-deductible.	State has instituted a per-drug cap on cost sharing that applies pre-deductible. Cap only applies to specialty-tier drugs; Or State has a total cap on Rx cost sharing that applies pre-deductible. Cap only applies to specialty-tier drugs.	State has instituted a total cap on Rx cost sharing that applies pre-deductible and for all prescription drugs.

Prescription Drug Cost Sharing (continued)

Results

Only nine states graded at A or B for this section, with the rest earning F's. This is because these are the only states to have enacted comprehensive legislation to limit cost sharing requirements for prescription drugs.

Unfortunately, most states that scored an F are not even considering legislation to limit cost sharing for prescription drugs. Some of these states do have limits of cost sharing for oral chemotherapy for cancers, but these restrictions do not apply to any other medication or condition. States that have enacted an prescription drug cap that did not earn a perfect score typically did not cap total costs, or the per-drug cap was high compared to other states.

Progress Since 2015

In 2015, two states (California and Colorado) adopted new rules limiting co-insurance for prescription drugs. Further progress on this issue has been limited because states are not willing to deal with the cost sharing issue without also dealing with controversial concerns around drug pricing. For instance, in many states where limits to drug cost sharing is being considered, opponents often force the legislature to simultaneously consider bills that would require manufacturers to disclose all of their manufacturing and marketing costs. Given the political obstacles, further progress on this issue may rely solely upon the work of state health agencies.

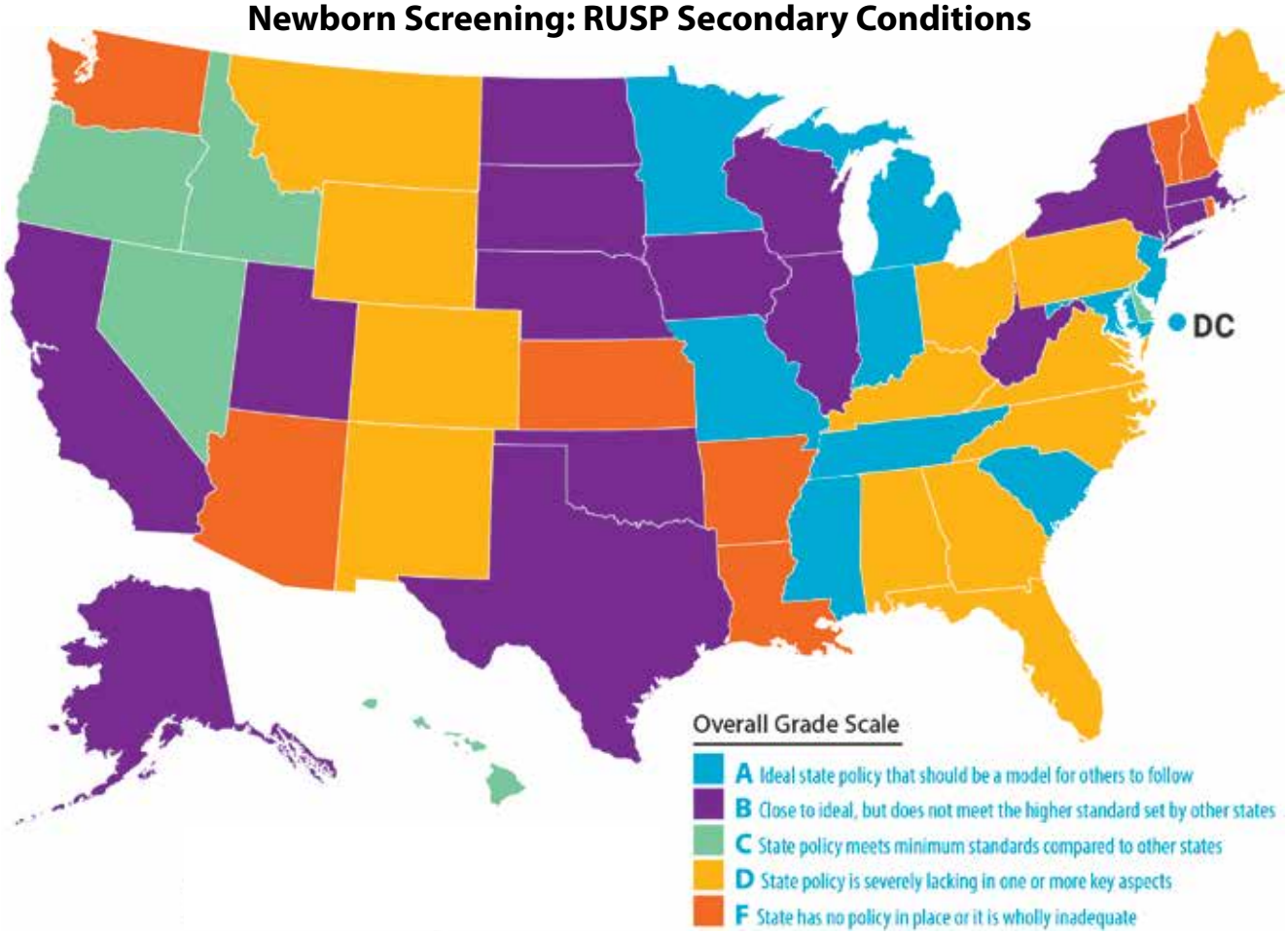
9 STATES SCORED AS AN A OR A B

41 STATES SCORED AN F

Newborn Screening

All 50 states have implemented screening of all core conditions on the Recommended Uniform Newborn Screening Panel (RUSP)

Newborn Screening: RUSP Secondary Conditions



Background

Newborn screening is one of the most successful public health programs ever enacted, saving tens of 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 their program to the state's specific needs. For example, states have great leeway in terms of what conditions to screen for or 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 Recommended Uniform Newborn Screening Panel (RUSP) developed by the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children[5], (Core & Secondary Conditions) 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 (continued)

NORD's Vision

Newborn screening saves lives. As a community, we cannot accept a reality in which a newborn baby in one state will be screened for a treatable disease that is not screened for in another. While changing this reality is not as simple as adding a new test to the screening list, it is NORD's goal to work with every state to ensure their newborn screening program reflects national recommendations.

Methodology

NORD graded states based on their implementation of screening for Core and Secondary Conditions on the RUSP. States were not graded on "non-recommended" conditions identified by the panel (see table 6). States were graded separately for the subcategories of Core Conditions and Secondary Conditions (see table 5).

For each individual condition, states were scored on a tiered rubric with possible scores of 0, 6, 8, or 10. A score of zero was utilized because many states do not cover certain recommended conditions at all. Page 23 lists non-recommended condition definitions; however, states were not graded on these conditions.

Table 3: Newborn Screening Scoring Rubric

TIER SCORE	DESCRIPTION
10	Condition is universally covered; Or State is implementing universal coverage
8	Screening is universally offered but not required; Or The Condition is detected as part of Multiple Reaction Monitoring[6]
6	Coverage is only mandated for select populations
0	Not covered

NEWBORN SCREENING HAS SAVED TENS OF THOUSANDS OF LIVES OVER THE PAST 50 YEARS

Newborn Screening (continued)

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		

Newborn Screening (continued)

Table 5: Newborn Screening: RUSP 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
CPT-1A	Carnitine palmitoyltransferase 1A
CPT-II	Carnitine palmitoyltransferase II deficiency
DE-RED	2,4 Dienoyl-CoA reductase deficiency
CA-II	Carbonic anhydrase II
MCKAT	Medium-chain ketoacyl-CoA thiolase deficiency
M/SCHAD	3-Hydroxyacyl-CoA dehydrogenase deficiency
SCAD	Short-chain acyl-CoA dehydrogenase deficiency
2M3HBA	2-Methyl-3-hydroxybutyric academia
2MBG	2-Methylbutyryl-CoA dehydrogenase deficiency
3MGA	3-Methylglutaconyl-CoA hydratase deficiency
Cbl-C,D	Cobalamin C cofactor deficiency
IBG	Isobutyrylglycinuria
MAL	Mal de Meleda
ARG	Argininemia
BIOPT-BS	Biopterin defect in cofactor biosynthesis
BIOPT-RG	Biopterin defect in cofactor regeneration
CIT-II	Citrullinemia, type II
H-PHE	Hyperphenylalaninemia
MET	Hypermethioninemia
TYR-II	Tyrosinemia, Type II
TYR-III	Tyrosinemia, Type III
GALE	Galactoepimerase deficiency
GALK	Galactokinase deficiency
HBS	HbS disease

Newborn Screening (continued)

Table 6: Newborn Screening: RUSP Non-Recommended Conditions

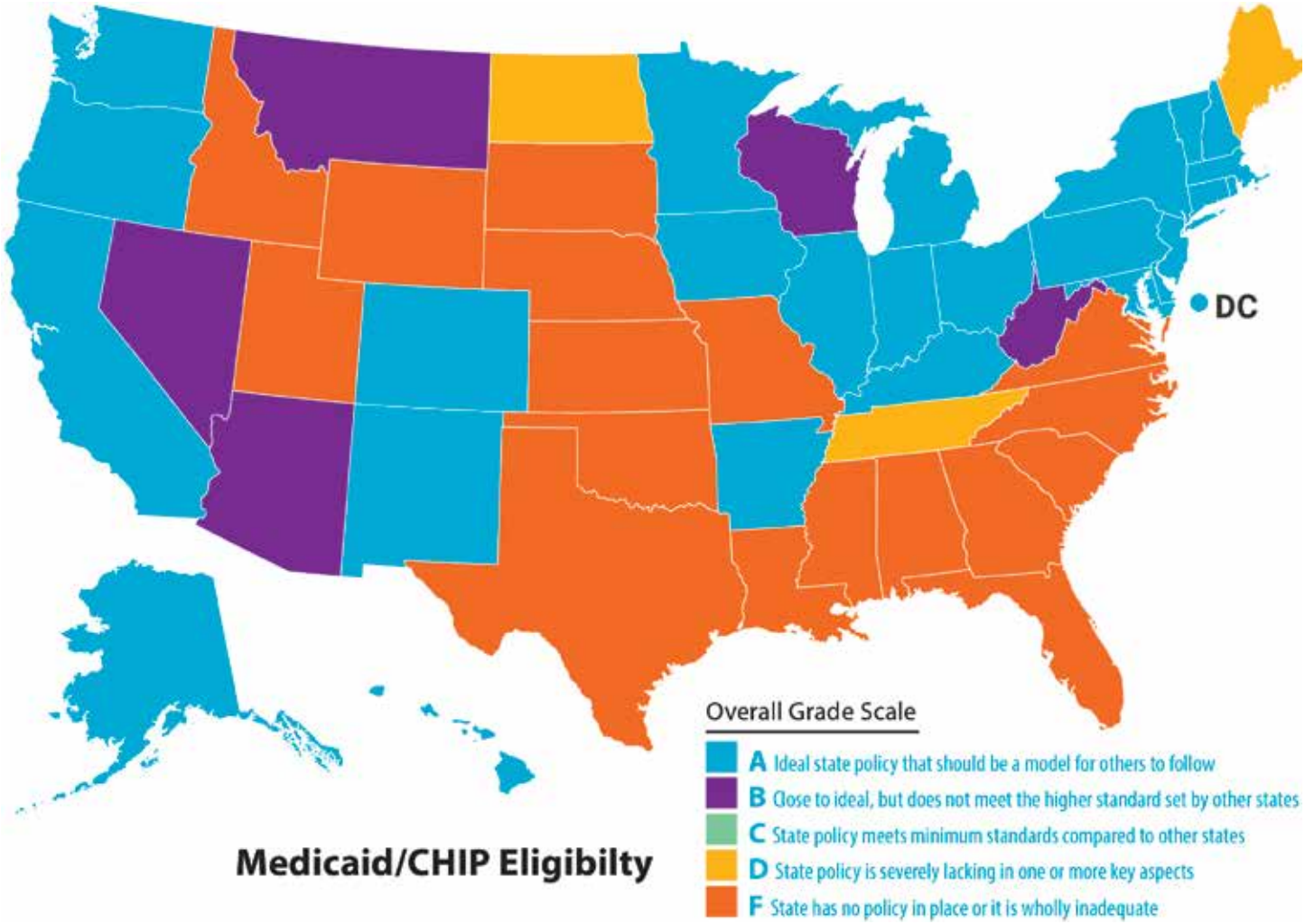
NEWBORN SCREENING: NON-RECOMMENDED CONDITIONS DEFINITIONS For more information on the diseases listed, visit NORD's Rare Disease Database or the National Institutes of Health	
HHH	Ornithine transcarbamylase deficiency
PRO	Prolinemia
EMA	Ethylmalonic encephalopathy
OTC,MTHFR	Ornithine transcarbamylase deficiency
NKH	Nonketotic hyperglycinemia
G6PD	Glucose-6-phosphate dehydrogenase deficiency
Krabbe	Leukodystrophy, Krabbe disease
Niemann-Pick	Niemann-Pick disease
Gaucher	Gaucher disease
Fabry	Fabry disease
MPS II	Mucopolysaccharidosis Type II
CPS	Carbamoyl phosphate synthetase I deficiency
5-OXO	Pyroglutamic acidemia
TOXO	Congenital toxoplasmosis

Results

All states earned grades of A for RUSP Core Conditions as nearly every state mandates screening for the full panel. However, there are a few areas of need that were not reflected in the overall grades. First, only a few states mandate screening of Adrenoleukodystrophy (ALD) and Mucopolysaccharidosis Type I (MPS I). Each of these disorders are relatively new additions to the RUSP Core Panel and it is expected that more states will require screening for ALD and MPS I moving forward. Second, there are still eight states that do not mandate screening for Severe Combined Immunodeficiency (SCID), which has been on the RUSP panel since 2010.

There is much greater variability in grades when it comes to state coverage of RUSP Secondary Conditions. For example, eleven states scored Fs for the panel because their screening programs only mandate coverage and reporting of a handful of Secondary Conditions or only do so for select populations. Overall, less than half of states scored an A or B for Secondary Conditions section.

Medicaid/CHIP Eligibility



Background

In 2012, the Supreme Court allowed states to choose whether or not to expand eligibility for their Medicaid program. Since the decision, a growing number of states have chosen to expand their Medicaid programs to cover all individuals at or below 138 percent of the federal poverty level (FPL is defined as $11,880 or less in annual income for a single person). 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 who 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/CHIP Eligibility (continued)

NORD's Vision

NORD believes every state can better serve the health care needs of the rare disease community by enabling more low-income adults to enroll in Medicaid. The federally-funded expansion provided by the Affordable Care Act is a good starting point, but we envision a future in which Medicaid is a true safety net for people in every state who cannot afford health insurance.

Methodology

Overall, states were graded across four subcategories: 1) eligibility for parents of dependent children; 2) eligibility for childless adults; 3) eligibility for pregnant women; and 4) eligibility for children (including CHIP-funded eligibility).

Each state was graded on a tiered rubric with possible scores of 0, 1, 6, 8, or 10 (see table 7). The only subcategory to include a score of zero was eligibility for childless adults because multiple states do not cover this population at all. The scores for each subcategory were combined to determine the overall category score.

The scoring criteria for children and pregnant women was determined in part by how states compare to each other. However, for the other subcategories (non-pregnant adults), the threshold for a perfect score was whether a state had expanded its Medicaid to 138% of the FPL.

Results

The number of states adopting the ACA-funded expansion of Medicaid has likely reached its end point for the foreseeable future. As a result, there will continue to be a gap in eligibility between states that have expanded eligibility for more adults (to 138% or more of the Federal Poverty Level) and those that have kept their adult eligibility at or near zero percent of FPL.

As it stands, states either scored high or poorly with no real middle ground. In total, 18 states scored a D or an F and the rest earned an A or a B.

Changes since 2015

There has not been significant changes to Medicaid eligibility since 2015. Moreover, the change in leadership at the White House coming in 2017 increases the chances that we could see certain aspects of the Affordable Care Act related to Medicaid altered or repealed completely.

32 STATES SCORED AN A OR B

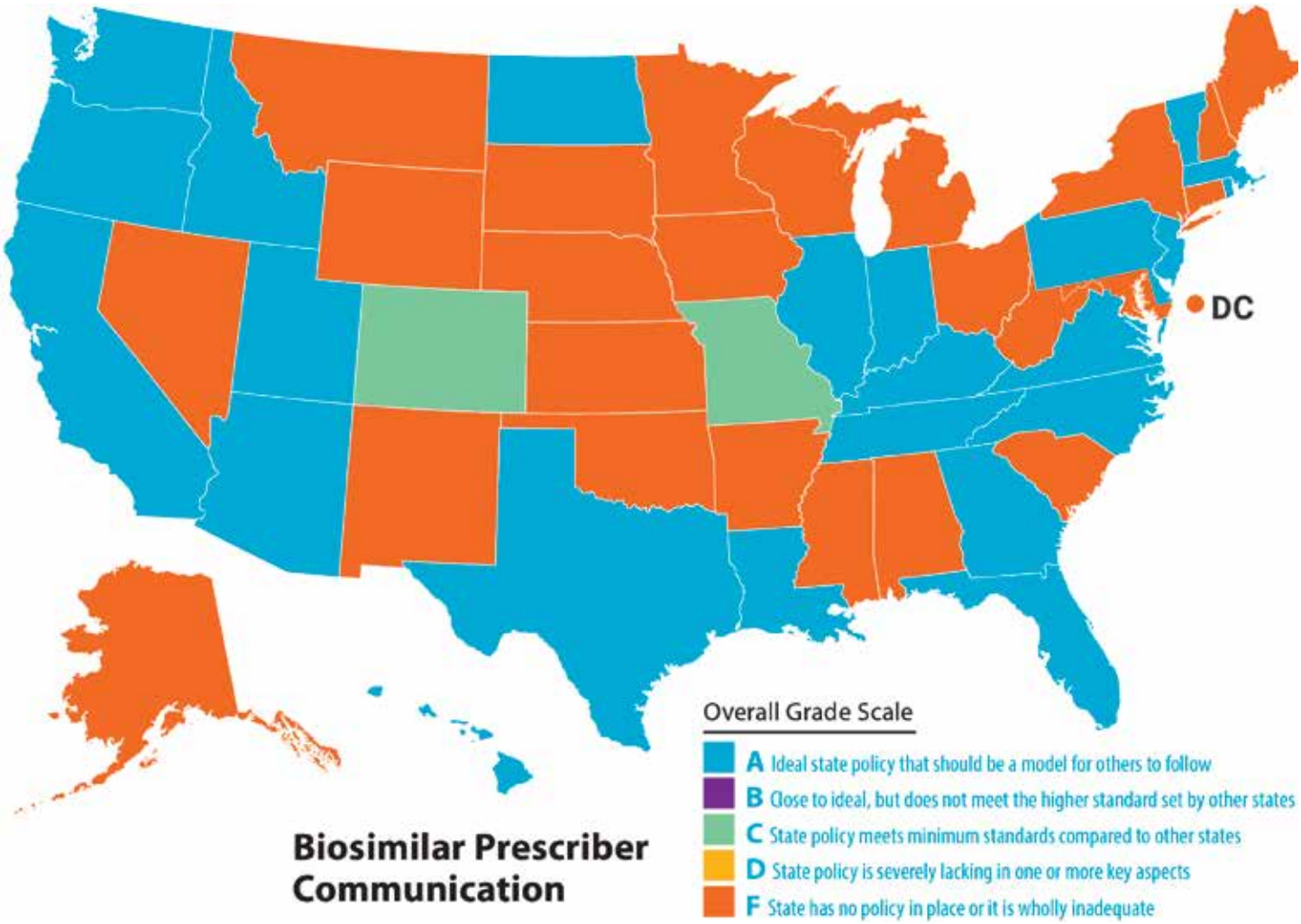
18 STATES SCORED A D OR AN F

Medicaid/CHIP Eligibility (continued)

Table 7: Medicaid/CHIP Eligibility Scoring Rubric

	TIER SCORE DESCRIPTION				
Sub-Category	**0**	**1**	**6**	**8**	**10**
Eligibility for Parents of Dependent Children	N/A	89% of the Federal Poverty Level (FPL) or less	90% to 99% of FPL	100%-137% of FPL	138% of FPL or greater
Eligibility for Childless Adults	No coverage	89% of the FPL or less	90% to 99% of FPL	100%-137% of FPL	138% of FPL or greater
Eligibility for Pregnant Women	N/A	Medicaid eligibility of 160% of FPL or less and no CHIP-funded eligibility	Medicaid eligibility of 161%-189% of FPL; Or Medicaid eligibility of at least 138% of FPL and CHIP-funded eligibility (or Separate CHIP) of 175% of FPL or greater	Medicaid eligibility of 190% - 219% of FPL; Or Medicaid eligibility of 161%-189% of FPL and CHIP-funded eligibility (or separate CHIP) of 200% of FPL or greater	Medicaid eligibility of 220% of FPL or greater; Or Medicaid >200% of FPL AND CHIP-funded eligibility (or separate CHIP) of 220% of FPL or greater
Eligibility for Children	N/A	No Medicaid eligibility or CHIP-funded eligibility for at least two age groups; Or Medicaid/CHIP eligibility less than 150% for all age groups.	Medicaid eligibility of 138%-150% of FPL for all age groups; Or Medicaid eligibility of 100% of FPL or greater for two age groups AND CHIP-funded eligibility (or separate CHIP) of 200% of FPL or greater for all age groups	Medicaid eligibility of 151% -199% of FPL for all age groups; Or Medicaid eligibility of at least 100% of FPL for all age groups AND CHIP-funded eligibility (or separate CHIP) of 200% of FPL or greater for all age groups	Medicaid eligibility of 200% of FPL or greater for all age groups; Or Medicaid eligibility of 175% of FPL or greater for all age groups AND CHIP-funded eligibility (or separate CHIP) of 220% of FPL or greater for all age groups

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 existing medicines 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 biologics 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.

Biosimilar Prescriber Communication (continued)

This has profound implications for how biologics are 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, doctors need to know which medicine was dispensed to the patient, whether a substitution was made and to what alternative product. These factors are all critical information that needs 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 December 2016, 31 states have passed laws requiring communications 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.

NORD's Vision

Patient-centered health care cannot be achieved if medical prescribing decisions can be overridden without the go-ahead from a patient or their doctor. NORD wants to see every state ensure that a biosimilar cannot be substituted without approval from a patient's prescribing doctor.

Methodology

In analyzing state policy pertaining to biosimilar prescriber communication, NORD focused on state polices that both required communication between the pharmacist and prescriber and allowed for the physician to override a substitution. State policy with both of these features earned an A (tier score of 10). States that required communication but did not provide an override earned a B (tier score of 8). There were no states that satisfied the criteria for a tier score of 6.

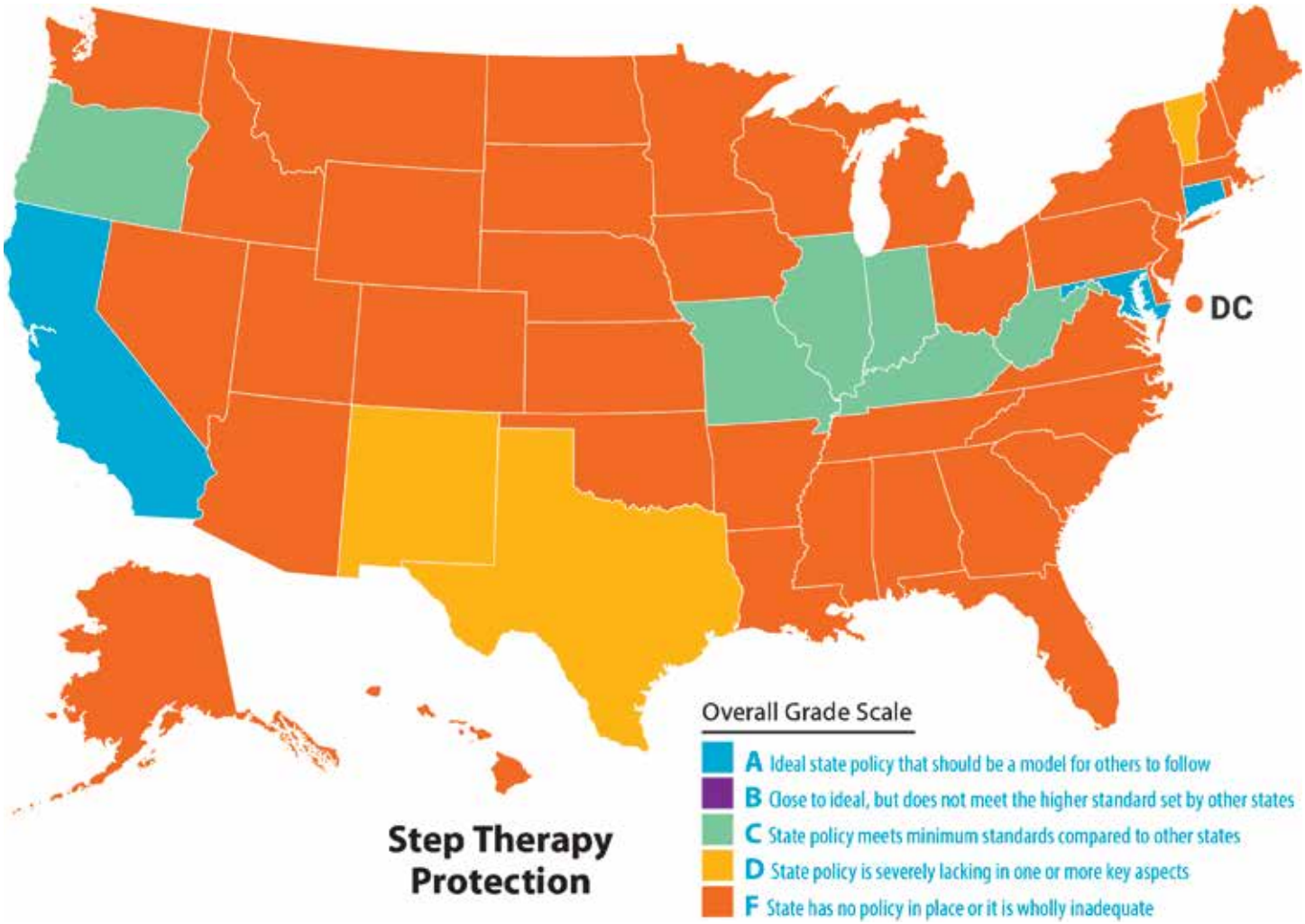
Results

The adoption of prescriber communication laws has been impressive. There are now 30 states with a law on the books requiring prescriber communication and allow for physician override. Only one state, Missouri, requires communication but does not allow for an override. All other states did not have a policy on the books and therefore earned F's. However, nearly every state without a policy will consider such legislation during the 2017 legislative session. Two of these states (Ohio and Michigan) could still pass new laws during the 2016 legislative session.

Table 8: Biosimilar Prescriber Communication

TIER SCORE	DESCRIPTION
10	Policy requires pharmacist to notify prescriber before making a substitution And Policy allows physician to override biosimilar substitution
8	Policy requires communication but does not allow for physician override
6	Policy includes prescriber communication but does not mandate it
0	No Policy

Step Therapy Protection



Background

Step therapy is a procedure by which insurers (public or private) require a patient to take one or more different medications before being put on the medicine preferred by their doctor. 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[7]), 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 go off a successful treatment and take a less effective medicine simply because it is also less expensive.

Step Therapy Protection (continued)

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

NORD's Vision

Step Therapy is another example of good intentions having unintended consequences. Health insurers use step therapy to ensure costs are controlled for both themselves and patients. However, there needs to be basic protections in place to prevent abuse of this system. NORD wants to ensure that every state has a policy in place preventing patients from needlessly going on medication that their doctor knows will not work for them.

Methodology

NORD evaluated 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 (reference above) earned a tier score of 10 (an A). States that do not allow for physician override earned a tier score of 8 (a B), and states that only require for patients to be informed earned a tier score of 6 (D). The full rubric can be found in Table 9.

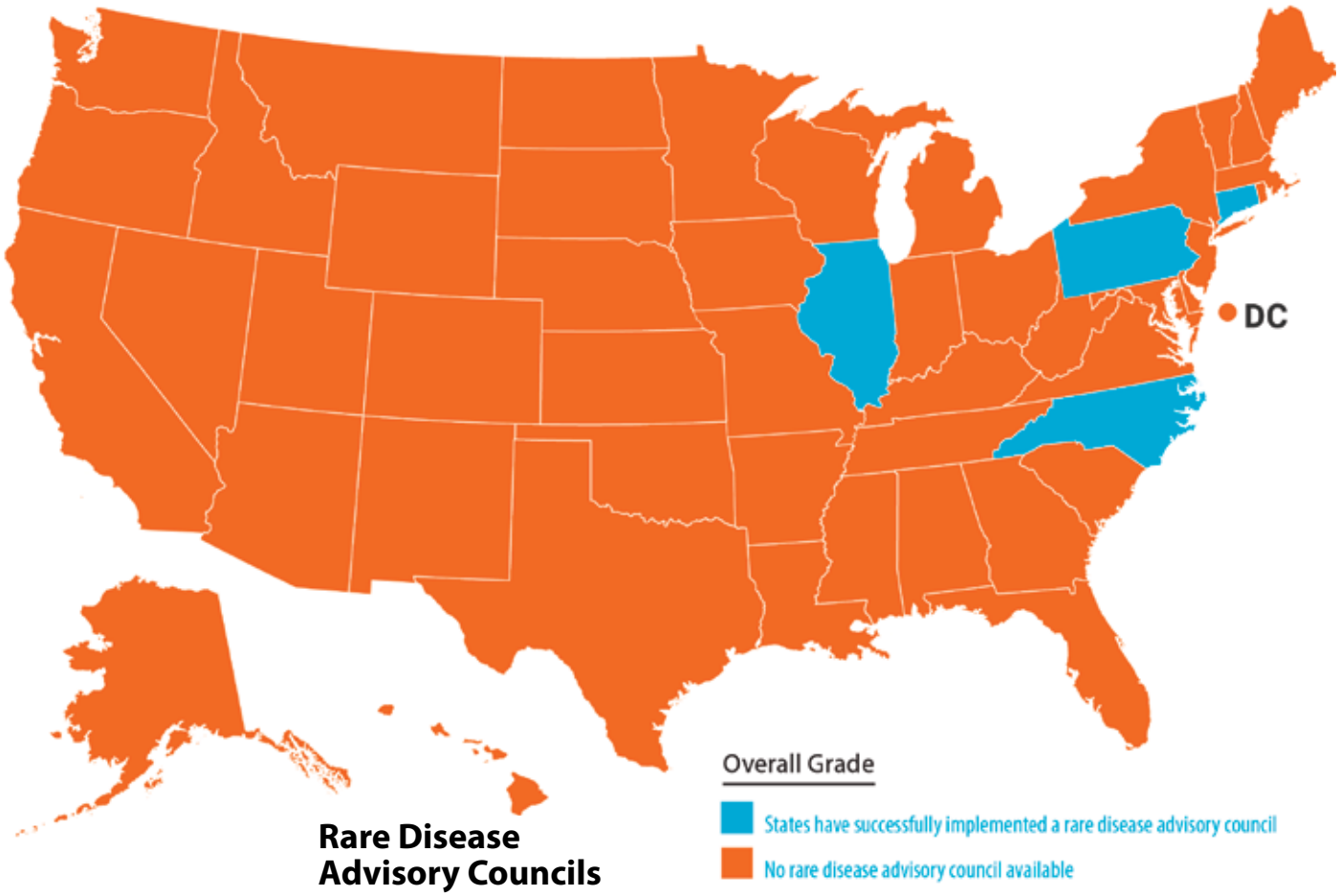
Results

Overall, 9 states earned an A or B in our assessment of step therapy. Nearly every state that has decided to take on this issue has done so in a comprehensive manner that address all of NORD's key criteria. Three states (New Mexico, Texas, and Vermont) scored D's in our assessment because their policy only required patients to be notified that step therapy would be implemented. All others states scored a failing grade because they had no policy at all.

Table 9: Step Therapy Scoring Rubric

TIER SCORE	DESCRIPTION
10	State requires step therapy to be based on independent medical criteria And State requires plans to establish a clear exceptions process for patients and doctors And State allows prescribing physician to over-ride step-therapy based on medical criteria
8	State requires step therapy to be based on independent medical criteria And State requires plans to establish a clear exceptions process for patients and providers
6	State only requires patients to be notified of the implementation of step therapy
0	No Policy

State Rare Disease Advisory Councils



Background

This report discusses in detail concrete policy changes states can make to ensure better access to medical care for rare disease patients. It is often the case that addressing these needs simply begins with ensuring that the rare community has a voice in state government. Several states have recognized the importance and value of this input 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 disseminating 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 strongly support the work of local advocates to create new councils in their state.

State Rare Disease Advisory Councils (cont'd)

NORD's Vision

There is not a one-size-fits-all approach to incorporating the rare patient voice into government. However, we have seen that the needs of the rare community will be overlooked if there is not a way to ensure our voices are heard. NORD's vision is for every state government to have a formal process in which our leaders are required to listen to the recommendations of rare disease experts and patients.

Methodology

Given the relatively recent onset of this policy issue, NORD did not grade states on this section for the 2016 report. A full grading across all 50 states will be included in the 2017 report.

Results

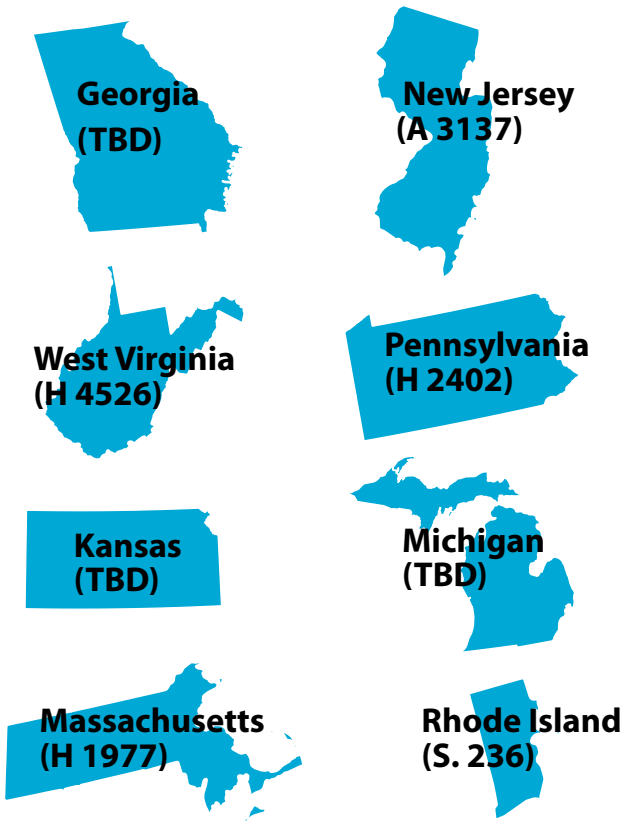
To date, the following states have successfully implemented a rare disease advisory council:

- **Connecticut**: In 2015, Connecticut established a Rare Disease Legislative Task Force based on HB 6580. This Task Force mandates broad participation from state government agencies, rare disease doctors and families, and CT-based universities.

- **Illinois:** In September 2016, Illinois created a new Rare Disease Legislative Commission after passing HB 4576. This commission mandates representation from rare disease families, doctors and state health agencies.

- **North Carolina:** In 2015, North Carolina created a Rare Disease Advisory Council by passing HB 823. The council serves to advise the Governor, the Secretary, and the General Assembly on research, diagnosis, treatment, and education relating to rare diseases and was created with broad support from NC-based universities.

The following states considered legislation to create a new council in 2016 or will take legislation in the 2017 legislative session:



SECTION III
APPENDIX

Appendix

To view and download full appendices for each issue addressed in the report card visit: rareaction.org/resources-for-advocates/state-report-card/

	Medical Foods		Prescription Drug Cost Sharing		Step Therapy Protection		RUSP Newborn Screening Core Conditions		RUSP Newborn Screening Secondary Conditions		Medicaid/Chip Eligibility		Biosimilars Prescriber Communication	
State	% Score	Grade	% Score	Grade	% Score	Grade	% Score	Grade	% Score	Grade	% Score	Grade	% Score	Grade
AL	40%	F	0%	F	0%	F	91%	A	60%	D	25%	F	0%	F
AK	65%	D	0%	F	0%	F	94%	A	81%	B	90%	A	0%	F
AZ	90%	A	0%	F	0%	F	92%	A	38%	F	85%	B	100%	A
AR	75%	C	0%	F	0%	F	94%	A	4%	F	90%	A	0%	F
CA	100%	A	80%	B	100%	A	97%	A	80%	B	90%	A	100%	A
CO	95%	A	80%	B	0%	F	93%	A	64%	D	95%	A	100%	A
CT	100%	A	0%	F	100%	A	97%	A	88%	B	100%	A	0%	F
DC	40%	F	80%	B	0%	F	94%	A	80%	C	95%	A	100%	A
DE	85%	B	0%	F	0%	F	93%	A	98%	A	90%	A	0%	F
FL	90%	A	10%	F	0%	F	94%	A	60%	D	43%	F	100%	A
GA	40%	F	10%	F	0%	F	93%	A	69%	D	48%	F	100%	A
HI	95%	A	0%	F	0%	F	93%	A	78%	C	90%	A	100%	A
ID	35%	F	0%	F	0%	F	92%	A	78%	C	20%	F	100%	A
IL	80%	B	0%	F	70%	C	100%	A	86%	B	90%	A	100%	A
IN	80%	B	0%	F	70%	C	91%	A	92%	A	90%	A	100%	A
IA	35%	F	0%	F	0%	F	94%	A	88%	B	95%	A	0%	F
KS	55%	F	0%	F	0%	F	90%	A	8%	F	38%	F	0%	F
KY	85%	B	0%	F	70%	C	96%	A	66%	D	90%	A	100%	A
LA	80%	B	80%	B	0%	F	91%	A	8%	F	25%	F	100%	A
ME	85%	B	100%	A	0%	F	93%	A	66%	D	60%	D	0%	F
MD	100%	A	80%	B	100%	A	92%	A	98%	A	95%	A	0%	F
MA	95%	A	0%	F	0%	F	90%	A	81%	B	90%	A	100%	A
MI	35%	F	0%	F	0%	F	94%	A	91%	A	90%	A	0%	F
MN	90%	A	0%	F	0%	F	94%	A	100%	A	100%	A	0%	F
MS	30%	F	0%	F	0%	F	93%	A	95%	A	43%	F	0%	F
MO	85%	B	10%	F	70%	C	97%	A	92%	A	43%	F	70%	C
MT	95%	A	80%	B	0%	F	94%	A	65%	D	85%	B	0%	F
NE	80%	B	0%	F	0%	F	93%	A	83%	B	43%	F	0%	F
NV	90%	A	0%	F	0%	F	91%	A	77%	C	85%	B	0%	F
NH	80%	B	0%	F	0%	F	93%	A	57%	F	95%	A	0%	F
NJ	95%	A	0%	F	0%	F	94%	A	100%	A	90%	A	100%	A
NM	90%	A	0%	F	60%	D	93%	A	68%	D	100%	A	0%	F
NY	85%	B	100%	A	0%	F	96%	A	84%	B	95%	A	0%	F
NC	40%	F	0%	F	0%	F	94%	A	60%	D	43%	F	100%	A
ND	70%	C	0%	F	0%	F	92%	A	88%	B	68%	D	100%	A
OH	50%	F	0%	F	0%	F	93%	A	60%	D	90%	A	0%	F
OK	45%	F	0%	F	0%	F	93%	A	83%	B	25%	F	0%	F
OR	100%	A	0%	F	70%	C	91%	A	76%	C	90%	A	100%	A
PA	70%	C	0%	F	0%	F	93%	A	62%	D	95%	A	100%	A
RI	90%	A	0%	F	0%	F	93%	A	19%	F	95%	A	100%	A
SC	45%	F	0%	F	0%	F	94%	A	96%	A	43%	F	0%	F
SD	75%	C	0%	F	0%	F	93%	A	88%	B	25%	F	0%	F
TN	75%	C	0%	F	0%	F	94%	A	94%	A	60%	D	100%	A
TX	90%	A	0%	F	60%	D	93%	A	81%	B	43%	F	100%	A
UT	90%	A	10%	F	0%	F	94%	A	82%	B	25%	F	100%	A
VT	95%	A	100%	A	60%	D	93%	A	56%	F	95%	A	0%	F
VA	60%	D	0%	F	0%	F	94%	A	62%	D	38%	F	100%	A
WA	70%	C	0%	F	0%	F	92%	A	46%	F	95%	A	100%	A
WV	45%	F	0%	F	70%	C	94%	A	82%	B	90%	B	0%	F
WI	50%	F	10%	F	0%	F	94%	A	80%	B	90%	B	0%	F
WY	95%	A	0%	F	0%	F	93%	A	64%	D	25%	F	0%	F

SECTION IV
RARE ACTION NETWORK℠

Take Action

The ultimate purpose of this report is to educate and empower advocates to make change in their state, and NORD's Rare Action Network℠ (RAN) is here to help. RAN is an advocacy network working to improve the lives of the 30 million Americans living with a rare disease. RAN serves a broad spectrum of stakeholders ranging from patients, to their families, caregivers, and friends; from researchers to industry; to physicians and academia. Through RAN, NORD has several new tools available to help you act on the 2016 State Report Card:

Join the Network

Members of the RAN are part of a 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.

Join Rare Action Network at www.rareaction.org.

Share your story with NORD

We want to hear your experiences living with a rare disease including how you have been affected by the issues covered in this report. Reach out to NORD's RAN team at action@rarediseases.org. You can also find Rare Action on Twitter at @RareAction.

Join a Rare Disease Day event

Rare Disease Day takes place on the last day of February each year. The purpose is to raise awareness with the general public and decision-makers about rare diseases and their impact on patients' lives.

You can make a difference, visit the Rare Action Network's state action center at www.rareaction.org to get involved in your state.

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2. HRSA, State Statutes and Regulations on Dietary Treatment of Disorders Identified Through Newborn Screening. 2014. Accessed online at: http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/reportsrecommendations/reports/statelaws.pdf
3. Nutrica Metabolics, State Coverage Database. Accessed online at http://www.medicalfood.com/Reimbursement/Coverage-by-State/by-State/

Prescription Drug Cost Sharing Sources

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2. National Psoriasis Foundation (www.psoriasis.org) analysis of step therapy implementation in the states. https://www.psoriasis.org/advance/step-therapy-states

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