

# 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 estimated 25-30 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 policy and regulatory decisions have been made. NORD 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). The ODA 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 drugs to treat to 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 brighten.

Following the enactment 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 idea that "Alone we are rare. Together we are strong.<sup>®</sup>" 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 in both the policy and research realms. Learn more about our work over the past 36 years at: *rarediseases.org/history.* 

In 2010, the enactment of the Affordable Care Act (ACA) addressed some of the issues that were most challenging for people with rare diseases, such as annual and lifetime caps on health care coverage. Many of the patients that we represent have benefited from the ACA, though, we know that there are still many issues to address. 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 fourth edition to demonstrate where progress has been made and where it is still needed. The 2019 State Report Card was compiled using data current as of January, 2019. This report reflects a snapshot in time, and more recent changes in state policy may not be reflected. The current political climate poses certain challenges for NORD and the rare disease community. We will continue to work with the current Administration, Congress, and all 50 states 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.

### **NORD Mission Statement**

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 280 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research and patient services.

### **Table of Contents**

- 4 Introduction
- 5 National Overview
- 12 Medical Nutrition
- 15 Prescription Drug Out-of-Pocket Cost Protections
- 17 Newborn Screening
- 20 Medicaid Eligibility and Waivers
- 24 Medicaid Home and Community-Based Services (HCBS) Waivers
- 26 Patient Protections for Step-Therapy (a.k.a Fail First)
- 28 Rare Disease Advisory Councils
- 30 Individual Insurance Market Protections
- 34 References
- 36 Special Thanks

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### Introduction

#### LIST OF POLICIES COVERED IN THIS REPORT

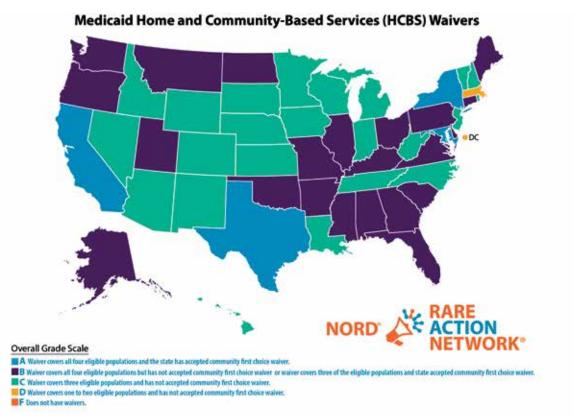
NORD analyzed and graded policy from every state and the District of Columbia (D.C.) across nine major issues affecting the rare disease community. They are as follows:

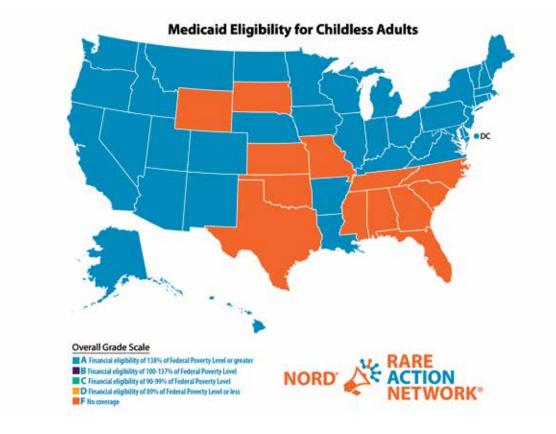
- 1. Medical Nutrition
  - a. Commercial coverage mandate
  - b. Commercial covered disorders
  - c. State-funded coverage mandate
  - d. State-funded covered disorders
- 2. Prescription Drug (Rx) Out-of-Pocket (OOP) Cost Protections
- 3. Newborn Screening (NBS)
  - a. Recommended Uniform Screening Panel (RUSP) core conditions
  - b. Use of dried blood spots (DBS)
  - c. Patient follow-up procedures
  - d. Adding new screens
  - e. Fee collection and use
  - f. NBS Advisory Committee
  - g. Statutory quality assurance
- 4. Medicaid Work Requirements and Cost Sharing (via waivers) Medicaid Eligibility:
  - a. Adult eligibility for childless adults
  - b. Adult eligibility for parent of a dependent child
  - c. Eligibility for pregnant women
  - d. Children's Health Insurance Program (CHIP) eligibility
- 5. Medicaid Home and Community-Based Services (HCBS) waivers
- 6. Step Therapy Protections (Fail First)
- 7. Rare Disease Advisory Councils
- 8. Individual Market Insurance Protections
  - a. Reinsurance
  - b. Regulation of short-term, limited-duration health
  - plans and association health plans
  - c. State-mandated pre-existing conditions protections

# SECTION I NATIONAL OVERVIEW

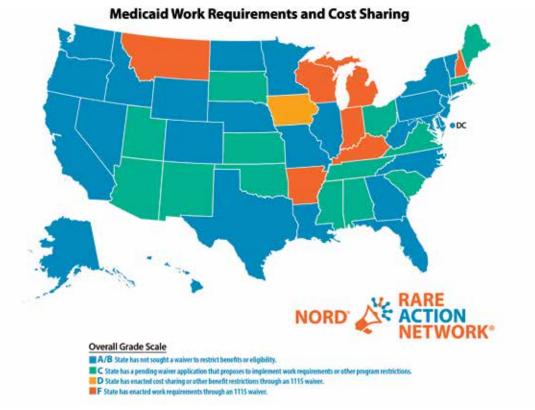


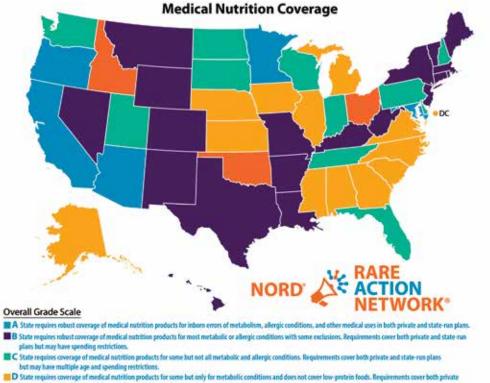
### **National Overview**







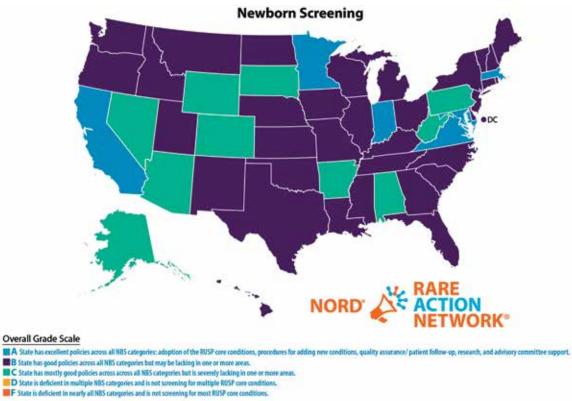


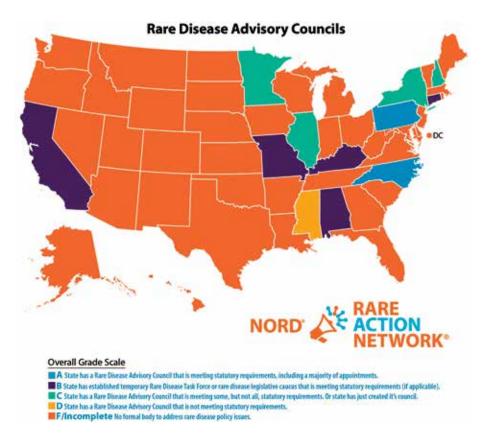


and state-run plans but may have multiple age and spending restrictions.

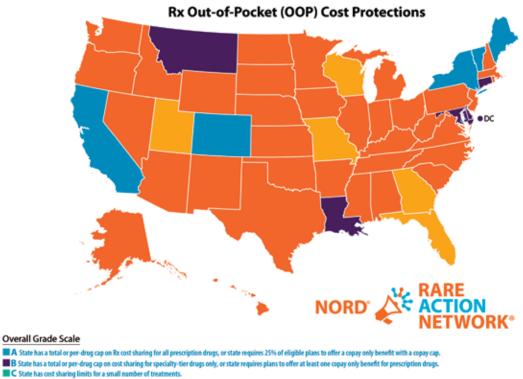
F State does not require coverage of medical nutrition products in either private plans or state-run plans.





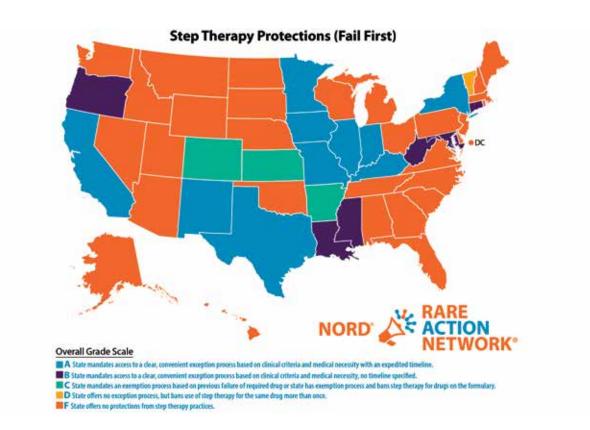




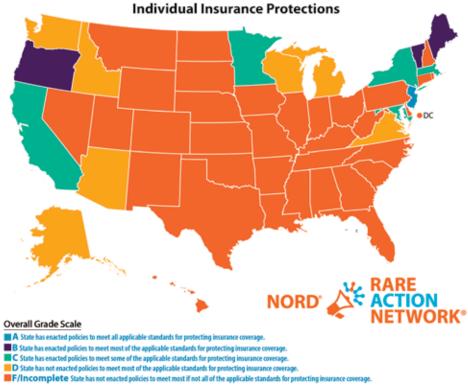


D State only limits cost sharing for chemotherapy.

F State does not have a cap on cost sharing.

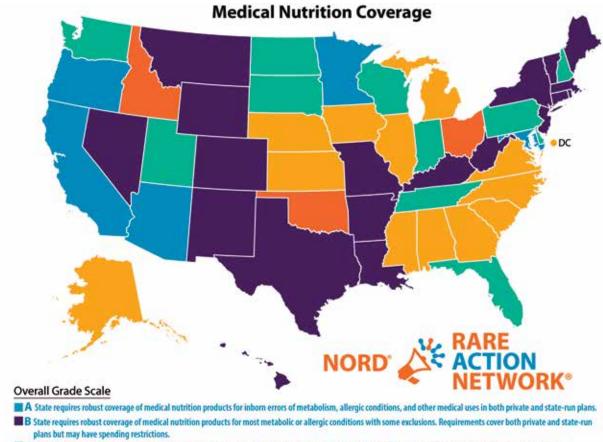






## **SECTION II** EXPLORING THE ISSUES





- C State requires coverage of medical nutrition products for some but not all metabolic and allergic conditions. Requirements cover both private and state-run plans but may have multiple age and spending restrictions.
- D State requires coverage of medical nutrition products for some but only for metabolic conditions and does not cover low-protein foods. Requirements cover both private and state-run plans but may have multiple age and spending restrictions.
- F State does not require coverage of medical nutrition products in either private plans or state-run plans.

Multiple rare disorders require special nutrition to prevent serious disability and allow for normal growth in children and adults. Effective medical nutrition may be the only viable treatment option available for patients living with these conditions.

The manufacturing of these medical nutrition products is highly specialized, making them more expensive for patients. For example, the average annual cost of formula for the metabolic disorder phenylketonuria (PKU) can be up to \$12,000, depending on factors such as age.<sup>1</sup> Coverage of medical nutrition 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 require coverage for a broader range of conditions. While much can be done at the federal level to increase access to medical nutrition, states also play an important role in ensuring access to these critical therapies.

Many states have mandated coverage of medical nutrition by eligible private plans sold within their state. However, in states without medical nutrition mandates, individuals needing these treatments are faced with paying out-of-pocket.

<sup>&</sup>lt;sup>1</sup>Buist, N., Huntington, K., Winter, S. *Healthcare Coverage for Medical Food Treatment of Inborn Errors of Metabolism*. National Organization for Rare Disorders. June 23. 2009. <u>https://journals.lww.com/topicsinclinicalnutrition/toc/2009/10000</u>

### **Medical Nutrition**



Despite the importance of medical nutrition for some rare disease patients, only some states mandate coverage through their Medicaid programs. For states that do not mandate coverage through Medicaid, a few have chosen to provide access to medical nutrition through other publicly-funded health programs or provide coverage on a case-by-case basis (which can lead to high variability in who gets access). Even in states that do mandate coverage of medical nutrition through Medicaid, there are often arbitrary limits based on cost, age, or gender.

Eligibility requirements for medical nutrition coverage are also concerning. Unfortunately, many states limit coverage (either in commercial insurance or in Medicaid) to certain disorders. Traditionally, most states have limited coverage to metabolic conditions.

More recently, however, states have begun to expand coverage to other conditions that require specialized nutrition. Disorders such as eosinophilic esophagitis or food protein-induced enterocolitis syndrome (FPIES) require highly specialized nutritional products in order to be properly treated. NORD supports medical nutrition coverage for any condition for which medical nutrition is a medically necessary component of effective treatment.

#### **GRADING METHODOLOGY**

The grading rubric for medical nutrition can be found on page 14. States were graded on four separate categories:

- 1. Coverage requirements for commercial health plans
- 2. Covered disorders requirements for commercial health plans
- 3. Coverage requirements for state-run programs
- 4. Covered disorders requirements for state-run programs

An overall state grade for medical nutrition coverage was determined by combining the grades for each of these four equally weighted categories.

States that placed age or monetary restrictions on coverage earned lower grades than states that had no such restrictions. Similarly, states with more covered conditions (ideally any condition for which medical nutrition is medically necessary) earned higher grades than states with fewer covered conditions.



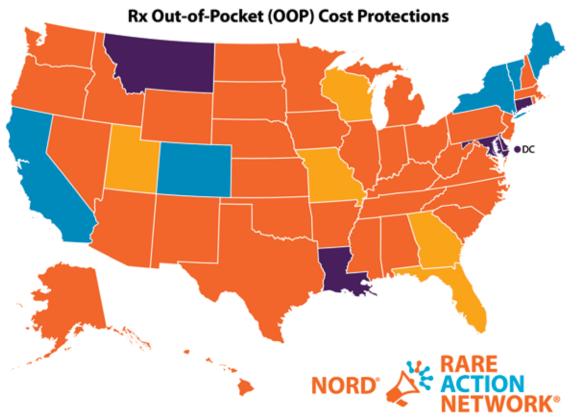
### **Medical Nutrition**

### Table 1: Medical Nutrition Grading Rubric

	DESCRIPTION			
GRADE	Coverage Requirements for Commercial Health Plans	Covered Disorders Requirements for Commercial Health Plans	Coverage Requirements for State-Run Programs	Covered Disorders Requirements for State-Run Programs
A	Coverage is required for both formula and low-protein nutrition with no limits on eligibility or coverage.	Covered disorders include all inborn errors of metabolism; eosinophilic disorders/ FPIES and other conditions requiring medical nutrition.	Mandated Medicaid coverage for medi- cal nutrition with no age or eligibility restrictions (or through a supplemental program).	Covered disorders include all inborn errors of metabolism; eosinophilic disorders/ FPIES; and other conditions requiring medical nutrition.
В	Coverage is required for formula and low-protein food but with age or dollar limits.	Covered disorders include all inborn errors of metabolism but exclude eosinophilic disorders/FPIES or other conditions re- quiring medical nutrition.	Mandated Medicaid coverage for formula and low-protein nutrition with restrictions (or through a supplemental program).	Covered disorders include all inborn errors of metabolism but exclude eosinophilic disorders/FPIES or other conditions requiring medical nutrition.
C	Coverage is required for both formula and low-protein nutrition but with age and dollar limits.	Covered disorders include three or more metabolic conditions, but exclude eosinophilic disorders/ FPIES and other medically necessary uses.	Coverage for formula and low-protein nutrition is on a case-by-case basis.	Covered disorders include three or more inborn errors of metabolism but exclude eosinophilic disorders/FPIES or other conditions requiring medical nutrition.
D	Coverage is required but with limits on eligibility (such as age) or coverage (such as a dollar cap or formula only).	Covered disorders include two or fewer metabolic conditions (such as PKU-only).	Mandated Medicaid coverage for formula but no coverage of low-protein nutrition.	Covered disorders include two or fewer metabolic conditions (such as PKU-only).
F	State does not mandate private insurance coverage of medical nutrition.	State does not mandate private insurance coverage of medical nutrition.	State does not mandate coverage for Medicaid. The state does not offer supple- mental programs to provide coverage.	State does not mandate coverage for Medicaid. The state does not offer supple- mental programs to provide coverage.

### **Prescription Drug Cost Sharing**





#### **Overall Grade Scale**

A State has a total or per-drug cap on Rx cost sharing for all prescription drugs, or state requires 25% of eligible plans to offer a copay only benefit with a copay cap.

B State has a total or per-drug cap on cost sharing for specialty-tier drugs only, or state requires plans to offer at least one copay only benefit for prescription drugs.

C State has cost sharing limits for a small number of treatments.

D State only limits cost sharing for chemotherapy.

F State does not have a cap on cost sharing.

Innovative new treatments are enabling rare disease patients to live healthier, happier lives. Unfortunately, however, the cost of these medicines can often be prohibitive. NORD recognizes that the high cost of drugs has a direct impact on patient access. Addressing this and other barriers to care is a priority for NORD. Further, we acknowledge the immense pressure that payors are under to control costs for the sake of all beneficiaries. Yet we do not believe that attempts to redress rising costs should come at the detriment of patients. Failing to support patients today, who are in need of immediate assistance to pay for their prescribed treatment, could have a negative impact on their health. Today, many insurers are resorting to methods that shift costs to patients, such as high deductibles and co-insurance, in an effort to sustain the overall healthcare system. In many instances, outof-pocket costs are outpacing wages, and patients are struggling.

For example, plans often require that enrollees pay co-insurance on prescriptions that can be as much as 50% of the actual cost of the medication.<sup>2</sup> For many people with a rare disorder, these costs can be untenable. As a consequence, patients in need of life-saving treatment are forced to go without their medication or use alternative treatments that are not as safe and effective. This type of cost sharing structure in health plans is occurring with increased frequency.

<sup>&</sup>lt;sup>2</sup> "Appleby, J. "Got Insurance? You Still May Pay A Steep Price For Prescriptions." Kaiser Health News. Published Oct 14, 2014. Accessed online at <a href="http://kaiserhealthnews.org/news/got-insurance-you-still-may-pay-a-steep-price-for-prescriptions/">http://kaiserhealthnews.org/news/got-insurance-you-still-may-pay-a-steep-price-for-prescriptions/</a>



### **Prescription Drug Cost Sharing**

For instance, in 2017, 84% of silver plans (the most common type of health insurance plan on the individual market) had a coinsurance requirement for specialty drugs.<sup>3</sup>

To assist patients in these difficult situations, several states have passed legislation mandating a limit on out-of-pocket costs for specialty medications. These limits range from \$100 to \$500 permonth, per-medication, depending on the type of plan. Thirdparty analysis has demonstrated that these types of limits on copays can be instituted with little to no impact on overall plan premiums for all beneficiaries.<sup>4</sup>

Other policy models do not apply per-drug caps on out-ofpocket costs but, rather, require that patients have the option of a "copay only" model when choosing a plan. These models can vary, but, generally, each insurance carrier must ensure that at least 25% of their plans at all levels include a copay only option wherein, in lieu of a deductible, the patient pays a flat copay each month that cannot exceed 1/12 of the plan's out-of-pocket maximum for the year.

In 2015, Colorado's Division of Insurance released a bulletin requiring plans to comply with a copay-only model.<sup>5</sup> Patients in Colorado now have the option of choosing a plan that offers greater control, predictability and easier financing.

#### **GRADING METHODOLOGY**

As noted above, when it comes to addressing the issue of high out-of-pocket costs, there are several different policies states can implement that have proven to be effective.

NORD gave states higher grades if they instituted caps for all drugs, whether per-drug or total. The grading rubric for this section can be found below.

#### **Table 2: Prescription Drug Cost Sharing Grading Rubric**

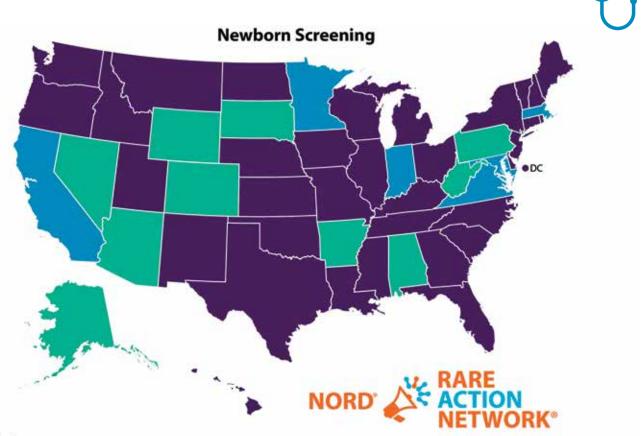
	DESCRIPTION
GRADE	Rx Cost Sharing Grading Rubric
A	State has a total cap or per-drug cap on Rx cost sharing that applies for all prescription drugs. OR The state requires 25% of eligible plans to offer a copay only benefit wherein the copay is capped.
В	State has a total cap or per-drug cap on cost sharing for specialty-tier drugs only. OR The state requires plans to offer at least one copay only benefit for prescription drugs.
c	State has cost sharing limits for a small number of treatments.
D	State only limits cost sharing for chemotherapy.
F	State does not have a cap on cost sharing.

<sup>&</sup>lt;sup>3</sup> Avalere PlanScape<sup>®</sup>, a proprietary analysis of exchange plan features, December 2016. Avalere analyzed data from the FFE Individual Landscape File released October 2016 and the California and New York state exchange websites.

<sup>&</sup>lt;sup>4</sup> "Impact of a \$150 Prescription DrugCost Sharing Cap on Silver Tier Individual Exchange Plans." Milliman, Inc. January 2017.

<sup>&</sup>lt;sup>5</sup> "Consumer Cost Share for Prescription Drug Benefits." Bulletin No. B-4.82. Released by Colorado Division of Insurance. January 2015. Accessed at <u>https://drive.google.com/file/d/0B7EeY5Lrg3\_qaWFEVzJFUWRYenc/view</u>

### **Newborn Screening**



#### **Overall Grade Scale**

- A State has excellent policies across all NBS categories: adoption of the RUSP core conditions, procedures for adding new conditions, quality assurance/ patient follow-up, research, and advisory committee support.
- B State has good policies across all NBS categories but may be lacking in one or more areas.
- C State has mostly good policies across across all NBS categories but is severely lacking in one or more areas.
- D State is deficient in multiple NBS categories and is not screening for multiple RUSP core conditions.
- F State is deficient in nearly all NBS categories and is not screening for most RUSP core conditions.

Newborn screening (NBS) programs throughout the United States have had great success at increasing the number of newborns screened at birth and, thereby, saving lives. Each year, approximately four million babies are screened through these programs. Of that four million, screening identifies over 12,000 infants each year with a disorder that, left undiagnosed and untreated, would cause severe developmental disability or death.<sup>6</sup> In many cases, newborn screening allows physicians to detect a heritable disease early enough to begin treatment before irreversible damage can occur. Newborn screening programs are typically regulated and operated at the state level, allowing each program to be customized to fit the state's specific needs. For example, states have flexibility in terms of the conditions screened and the use of samples following a blood spot test. The strength of a state's NBS program, however, is not limited to the number of conditions detected. Funding of the program, follow-up guidelines, quality assurance, the use of the remaining dried blood spots (DBS), the existence and structure of an advisory committee, and the process by which states can add new conditions to its program are also important characteristics. If a condition is added without proper quality assurance, follow-up programs or expert recommendation, there could be a surge in inaccurate screening results (false-positives or false-negatives), creating the potential for confusion and fear among patients and their families. NORD expanded its analysis of NBS in this report to better capture these issues and compare state programs to each other.

<sup>&</sup>lt;sup>6</sup> <u>https://www.nichd.nih.gov/health/topics/newborn/conditioninfo/infants-screened</u>

### **Newborn Screening**

#### **GRADING METHODOLOGY**

NORD supports robust, well-funded newborn screening programs in every state. NORD encourages state lawmakers to work with their health department to prioritize the state's newborn screening program and the early detection of these potentially debilitating diseases. NORD also encourages every state to adopt the Recommended Uniform Screening Panel (RUSP) developed by the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) and will continue to advocate for its adoption in each state that currently does not screen for all the disorders included on the panel.

The grading rubric for this section can be found on page 19. States were graded separately on the following seven separate categories, and an overall state grade for NBS was determined by taking the average of these seven separate grades:

- Screening for RUSP core conditions: NORD considered how many RUSP core conditions are on a state's NBS panel using the state's relevant statute and regulation, the NewSTEPSs Data Repository and Baby's First Test.
- 2. Adding RUSP core conditions: States should have a procedure to add conditions to RUSP panels in an efficient and appropriate manner without unnecessary barriers. NORD assessed states' relevant statutes and regulations as well as the NewSTEPSs Data Repository to determine what processes exist to adopt conditions.
- 3. Funding: NBS programs require funding for everything from laboratory personnel to equipment. Health departments should be permitted to independently set newborn screening fees to meet the needs of the programs, and such funds should be used only to improve the programs. NORD assessed states' relevant statutes and regulations as well as the NewSTEPSs Data Repository to determine how states fund their newborn screening programs.
- 4. Using Dried Blood Spot (DBS): The DBS that remain following screening of an infant are an invaluable source of research data on not only the diseases

covered by NBS programs but also for a host of other conditions. Use of DBS generally falls into three categories: (1) DBS are used for quality assurance and quality control (QA/QC) purposes, such as to verify the results of other NBS tests; (2) 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 (3) DBS are 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. In all three scenarios, the DBS are de-identified, or stripped of anything that could link them to the infant. In grading this section, NORD used states' relevant statutes and regulations as well as the NewSTEPSs Data Repository to evaluate states' policies for the use of DBS. NORD encourages states to retain DBS and use them for research and quality assurance.

- 5. Following-up: Once a baby has been screened, it is critical that states have programs to guide the baby and the parents. For example, if a screen comes back positive, the state needs to be prepared to get that information to the parents, explain what it means, take care of the baby, and connect the family to appropriate resources in a timely fashion. In reviewing this section, NORD used states' relevant statutes and regulations as well as the NewSTEPSs Data Repository to determine the extent of a state's follow-up program.
- 6. Quality: Quality in NBS programs is critical. Any slight adjustment or miscalculation can result in screens failing to identify potentially fatal conditions. Therefore, is it crucial that a state have programs in place to ensure that its NBS laboratories are engaging in adequate quality assurance and quality control (QA/QC). For this section, NORD used states' relevant statutes and regulations as well as the NewSTEPSs Data Repository to ascertain existing quality requirements.

### **Newborn Screening**

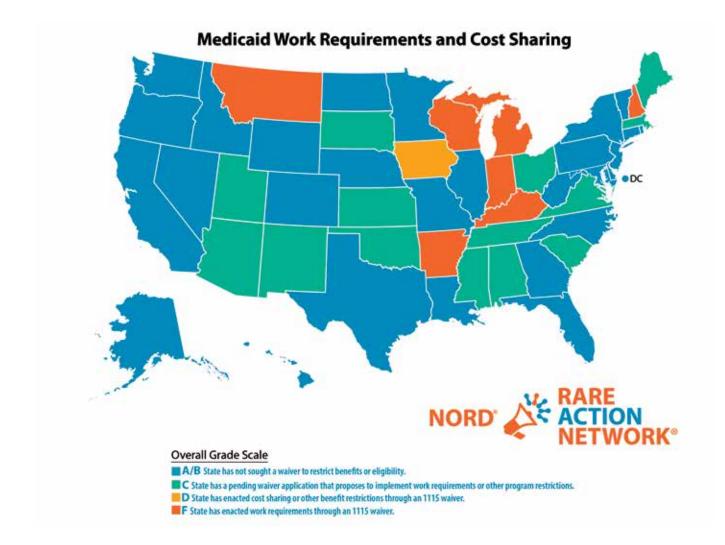


 Advisory committee: It is important that states' NBS programs have an advisory committee comprised of experts in the field, including laboratory personnel who can make recommendations on how to improve the program, and that such advisory committees meet at least once a year. In analyzing this section, NORD used states' relevant statutes and regulations as well as the NewSTEPSs Data Repository to identify whether the state had an advisory committee and, if so, how it operates.

	DESCRIPTION						
GRADE	Screening for RUSP Core Conditions	Adding RUSP Core Conditions	Funding	DBS Use	Follow-Up	Quality	Advisory Committee
A	Screens for all core conditions	Conditions are added automatically	NBS program has a dis- tinct stream of revenue AND health department can easily set fee	Uses for research and for QA/QC	Has a robust short-term and long-term program in place with funding	Has an excellent program in place OR has a good program in place with funding	Has an entity that includes a diverse membership AND meets more than once a year
В	Up to three condi- tions for which it does not screen	Health department can easily add conditions on its own	NBS program has a dis- tinct stream of revenue OR health department can easily set fee	Uses for research and QA/QC with consent	Has a short-term and long-term program	Has a good program in place	Has an external entity that meets more than once a year
C	Four to five condi- tions for which it does not screen	Health department can add conditions on its own	Revenue comes from general funds and it is hard to change fee OR there are supplemental appropriations (e.g., Title V)	Uses for QA/QC	Has a short-term program	Has a program in place	Has an external entity that only meets once a year
D	More than five conditions for which it does not screen	Legislature must approve the addition of conditions	The NBS fee and the resulting revenue are subject to the legislature	Stores, but does nothing AND permits parents to destroy	Has some educational materials	Does not have a program OR only focuses on specimen collection	Does not have an external entity but has an internal entity
F	No screening	State does not add conditions	No funding	Does nothing and destroys in under a year	Does not have anything	Low quality	Does not have anything

### **Table 3: Newborn Screening Grading Rubric**





In 2012, the Supreme Court decision in *National Federation* of Independent Business v. Sebelius enabled states to choose whether to expand the financial eligibility for their Medicaid program.<sup>7</sup>Since that decision, a growing number of states have decided to expand their Medicaid programs to cover all individuals with incomes at or below 138% of the federal poverty level (FPL). Such expansion has resulted in an increase of access to needed health services and allowed thousands of Americans with rare diseases to gain health insurance coverage. States that have opted not to expand their eligibility have left millions of Americans without health insurance who would otherwise be eligible for Medicaid coverage. The State Children's Health Insurance Program (CHIP) is also an important source of health coverage for children and families that are ineligible for traditional Medicaid. All states provide some degree of coverage for children and families through CHIP but may operate such programs 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.



In an attempt to control health care costs and improve services for Medicaid beneficiaries, states have sought section 1115 waivers that enable them to make substantial changes to Medicaid benefits and eligibility.

Section 1115 waivers enable states to administer demonstration projects that have been approved by the Centers for Medicare and Medicaid Services (CMS). These projects waive certain Medicaid requirements and allow a state to direct federal Medicaid funds in ways that would otherwise not be permitted under federal law. These waivers are supposed to align with the objectives of the Medicaid program, but 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.

For example, many states have proposed adding work requirements to their Medicaid programs. NORD does not believe that Medicaid coverage should be dependent on work. There is no evidence to suggest that this policy would improve the lives of beneficiaries in the way that many states argue. On the contrary, the evidence suggests that those Medicaid beneficiaries who are able to work largely already do, and those same beneficiaries, in addition to those unable to work, would be at risk of losing coverage under a work requirement due to the difficulties of implementation.<sup>8</sup>Some state proposals include exemptions to the work requirements, however, such exemptions are not likely to capture every deserving Medicaid beneficiary. Given the 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.

States are also debating a number of proposals to increase cost sharing and eliminate retroactive eligibility, which could leave some individuals, who may be below FPL, with extensive medical debt. Such proposals could be detrimental to all Medicaid beneficiaries, including individuals with rare diseases.

These concerns are not exhaustive, but they are representative of the ways in which the rare disease community may 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 contrary to the goals of the Medicaid program, but could also worsen healthcare outcomes and increase costs for rare disease patients and their caregivers.

<sup>&</sup>lt;sup>8</sup> Benjamin D., Sommers, M.D., Ph.D. "Medicaid Work Requirements—Results from the First Year in Arkansas." New England Journal of Medicine. June 19, 2019. Accessed at: <u>https://www.nejm.org/doi/full/10.1056/NEJMsr1901772</u>)



#### **GRADING METHODOLOGY**

The grading rubrics for Medicaid financial eligibility and section 1115 waivers can be found on pages 22-23. States were graded on each of the following categories (there is no overall Medicaid grade):

- Eligibility for Parents of Dependent Children: NORD analyzed the income level (%FPL) at which states allow parents of dependent children to enroll in Medicaid.
- 2. Eligibility for Childless Adults: NORD assessed whether states have expanded their Medicaid program for childless adults. States that have not expanded their Medicaid programs do not allow childless adults to enroll in Medicaid, regardless of their income.
- 3. Eligibility for Pregnant Women: NORD assessed state financial eligibility requirements for pregnant women to enroll in Medicaid. All states have some eligibility for pregnant women to enroll in Medicaid (or CHIP), but the income eligibility can vary widely.
- 4. Eligibility for Children (Including CHIP-Funded Eligibility): NORD assessed state financial eligibility requirements for children ages 0-18. All states have some eligibility allow for children to enroll in Medicaid (or CHIP) but income eligibility can vary widely.
- 5. Section 1115 Waivers: Our analysis of section 1115 waivers focused on whether states have implemented or are pursuing work requirements and other restrictions within their Medicaid program. States that have implemented these restrictions earned a "D" or "F" grade, and states that are currently pursuing a waiver earned a "C" grade.

#### Table 4: Medicaid Section 1115 Waivers Grading Rubric

	DESCRIPTION		
GRADE	Medicaid Section 1115 Waivers Grading Rubric		
A/B	State has not sought a waiver to restrict benefits or eligibility.		
c	State has a pending waiver application that proposes to implement work requirements and/or other program restrictions.		
D	State has enacted cost sharing or other benefit restrictions through a section 1115 waiver.		
F	State has enacted work requirements through a section 1115 waiver.		

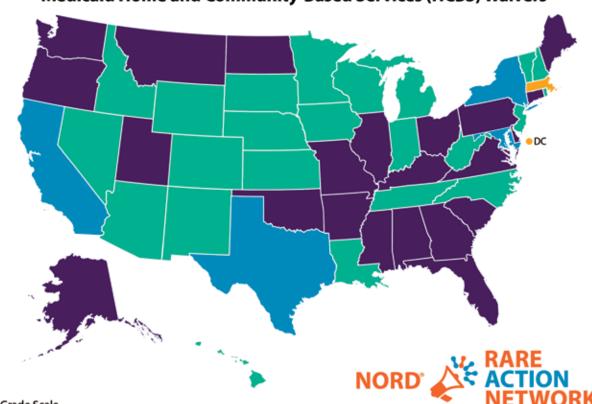


### Table 5: Medicaid Financial Eligibility Grading Rubric

	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 or CHIP eligibility of 220% of FPL or greater	Medicaid or CHIP eligibility of 300% of FPL or greater for all age groups
В	100%-137% of FPL	100%-137% of FPL	Medicaid or CHIP eligibility of 190% to 219% of FPL	Medicaid or CHIP eligibility of 195% to 299% of FPL for all age groups
C	90% to 99% of FPL	90% to 99% of FPL	Medicaid or CHIP eligibility of 150% to 189% of FPL	Medicaid or CHIP eligibility of 150% to 194% of FPL for all age groups
D	89% of FPL or less	89% of FPL or less	Medicaid or CHIP eligibility of 149% of FPL or less	Medicaid or CHIP eligibility of up to 150% of FPL for all age groups
F	No coverage	No coverage	No coverage	No coverage



### **Home and Community-Based Services**



### Medicaid Home and Community-Based Services (HCBS) Waivers

#### Overall Grade Scale

A Waiver covers all four eligible populations and the state has accepted community first choice waiver.

B Waiver covers all four eligible populations but has not accepted community first choice waiver or waiver covers three of the eligible populations and state accepted community first choice waiver.
C Waiver covers three eligible populations and has not accepted community first choice waiver.

D Waiver covers one to two eligible populations and has not accepted community first choice waiver.

Persons with a rare disease who have a severe disability often require intensive long-term care and support services above and beyond what is needed to manage their underlying medical condition. These services, known formally as "long-term services and supports" (LTSS), include skilled nursing care, transportation, assistance with activities of daily living, and medication management. For most families and caregivers, acquiring and paying for long-term care is a significant burden that they bear despite its challenges. Fortunately, nearly all states have developed programs that provide coverage for a comprehensive set of LTSS to eligible individuals.<sup>9</sup> While these programs can never fully supplant the role and responsibility of families and caregivers, the coverage they provide is essential.

In the past, LTSS required enrollees to obtain care in an institutional setting, such as a nursing home. However, since 1999, when the Supreme Court ruled in Olmsted v. L.C. that unjustified segregation of persons with disabilities constitutes discrimination in violation of the Americans with Disabilities Act (ADA), the availability of LTSS in home and community-based settings has proliferated.<sup>10</sup>

F Does not have waivers.

<sup>&</sup>lt;sup>9</sup> Reaves EL, Musumeci MB. Medicaid and Long-Term Services and Supports: A Primer. The Henry J. Kaiser Family Foundation. <u>https://www.kff.org/medicaid/report/medicaid-and-long-term-services-and-supports-a-primer/. Published June 28, 2017. Accessed March 18, 2019.</u>

<sup>&</sup>lt;sup>10</sup> Olmstead v. L. C., 527 U.S. 581 (1999)

### **Home and Community-Based Services**



Today, every state has obtained a Home and Community-Based Services (HCBS) waiver (or a similar type of waiver) to provide LTSS to persons with disabilities.

The use of these waivers is a welcome coverage model for the rare disease community, however, not all states use these waivers to cover all who require LTSS. For example, several states exclude persons with a physical disability from their HCBS program. Others have not adopted the newer Community First Choice state plan option (1915(k)), a state option that provides enhanced federal funding for LTSS and helps remove waiting lists, allowing states to expand access and more fully integrate care services into their Medicaid program. NORD believes that all states should use the waiver process to ensure access to Home and Community-Based Services for all four eligible populations: persons with a physical disability, persons with an intellectual/ developmental disability, aged persons with a disability and children with a disability.

#### **GRADING METHODOLOGY**

The grading rubric for Home and Community-Based Services waivers can be found below. States were given an overall grade for this category based on an analysis of the following criteria:

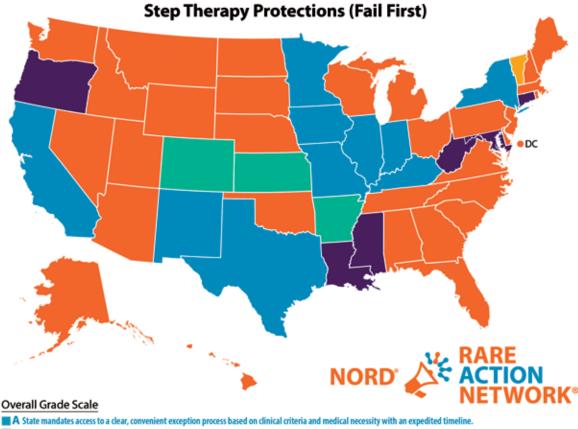
- 1. Whether the state has a HCBS waiver through either the 1915(c) or 1115 Medicaid LTSS authority;
- 2. Whether and what populations are covered by the waiver (covered populations include persons with a physical disability, persons with an intellectual/ developmental disability, aged persons with a disability, children with a disability); and
- 3. Whether the state has accepted the 1915(k) state plan option.

	DESCRIPTION			
GRADE	Does the state have a HCBS waiver through either the 1915(c) or 1115 Medicaid LTSS authority?	What populations are covered by the waiver?	Has the state accepted the 1915(k) state plan option?	
A	Yes	Persons with a physical disability, persons with an intellectual/developmental disability, aged persons with a disability and children with a disability	Yes	
В	Yes	All four populations	No	
В	Yes	Three of the populations	Yes	
c	Yes	Three of the populations	No	
D	Yes	One to two of the populations	No	
F	No	None	No	

#### Table 6: Home and Community-Based Services Waivers Grading Rubric



### **Patient Protections for Step Therapy**



B State mandates access to a clear, convenient exception process based on clinical criteria and medical necessity, no timeline specified.

C State mandates an exemption process based on previous failure of required drug or state has exemption process and bans step therapy for drugs on the formulary.

C state mandates an exemption process based on previous failure or required orug or state has exemption process and bans step therapy for drugs on the D State offers no exception process, but bans use of step therapy for the same drug more than once.

State offers no exception process, but bans use of step therapy for the same drug

F State offers no protections from step therapy practices.

In an effort to contain costs, many insurers have instituted "step therapy" programs, also known as "fail first," under which insurers (public or private) require a patient to take one or more alternative medications before permitting patients to access the medicine prescribed by their provider. While this is done by insurers as an attempt to control healthcare costs, step therapy has been increasingly applied to patients with little regard to their medical situation or treatment history. As a result, step therapy requirements can delay appropriate treatment and ultimately increase healthcare costs, not lower them.

As the use of step therapy has increased (at least 60% of commercial health plans have implemented it), so has the need for states to ensure that these requirements do not interfere with appropriate care for patients.<sup>11</sup>For example, 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.

<sup>&</sup>lt;sup>11</sup> Pharmaceutical step-therapy interventions: a critical review of the literature. J Manag Care Pharm. 2011 Mar;17(2):143-55.

### **Patient Protections for Step Therapy**



Some states have instituted protections around the use of step therapy to ensure patients obtain the care and treatment they need at the right time. In general, these protections:

- 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 controls protect patients while still enabling health plans to achieve the cost-saving benefits of step therapy when appropriate.

#### **GRADING METHODOLOGY**

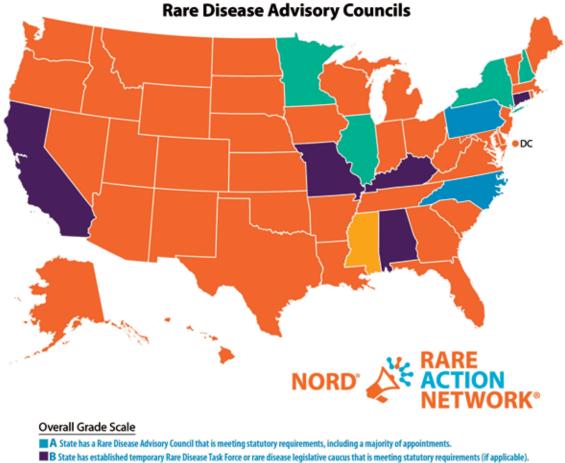
NORD assessed states based on the three protections outlined above using the grading rubric on this page.

#### Table 7: Step Therapy Grading Rubric

	DESCRIPTION
GRADE	Step Therapy Grading
A	State mandates access to a clear, convenient exception process based on clinical criteria and medical necessity with an expedited timeline
В	State mandates access to a clear, convenient exception process based on clinical criteria and medical necessity, no timeline specified
c	State mandates an exemption process based on previous failure of required drug, or state has exemption process and bans step therapy for drugs on the formulary
D	State offers no exception process, but bans use of step therapy for the same drug more than once
F	State offers no protections from step therapy practices



### **State Rare Disease Advisory Councils**



- C State has a Rare Disease Advisory Council that is meeting some, but not all, statutory requirements. Or state has just created it's council.
- D State has a Rare Disease Advisory Council that is not meeting statutory requirements.
- F/Incomplete No formal body to address rare disease policy issues.

In 2015, rare disease patients, families, caregivers and providers in North Carolina came together to create the first Rare Disease Advisory Council (RDAC) to give their community a stronger voice in government. Since then, other advocates have sought advisory councils in many states. With the support of NORD and other patient organizations, RDACs are enabling each of these states to address barriers that prevent individuals living with a rare disease from obtaining proper treatment and care for their condition.

With over 7,000 known rare diseases, it is difficult for state policymakers to have an in-depth understanding of the entire rare disease community. This lack of awareness contributes to common difficulties that rare disease patients face every day, such as delays in diagnosis, misdiagnosis, lack of treatment options, high drug costs and limited access to medical specialists.

Although research into rare diseases is advancing and producing new breakthrough treatments for patients, state policies affecting patient access to these breakthroughs are often determined without consulting individual disease communities. Without greater representation in state government of the rare disease community, legislators and other officials cannot adequately address this problem and other barriers to better care for this population.

### **State Rare Disease Advisory Councils**



RDACs help address the needs of the rare disease community within a state by giving patients, families, caregivers and other stakeholders an opportunity to make formal recommendations to state leaders about the most important issues they face. The membership of rare councils includes a variety of stakeholders who represent the rare disease community, including patients, caregivers, doctors, insurers, drug manufacturers and researchers.

Based on feedback from advocates in several states, NORD has identified key features of how a rare disease advisory council should complete its mission and how it should be structured. First and foremost, it is critical that councils include stakeholders from across the rare disease community. Currently, every disease council has a similar membership that includes the following representatives:

- Health department officials;
- Elected legislative officials;
- Academic researchers;
- Health providers (physicians, nurses, geneticists, pharmacists, etc.);
- Hospital administrators;
- Patients and caregivers; and
- Health industry representation (drug manufacturers, insurance companies, etc.).

The purpose of a council is to act as an advisory body on rare diseases to the governor, legislature and state agencies, as well as other important stakeholders (such as state universities). Currently, every RDAC is required to report back to the governor and state health department on its activities and to make specific recommendations to improve public policy.

Councils typically meet throughout the year and convene public hearings, consult experts and conduct informal research. The ultimate goal of this work is to develop policy recommendations and best practices to share widely.

#### **GRADING METHODOLOGY**

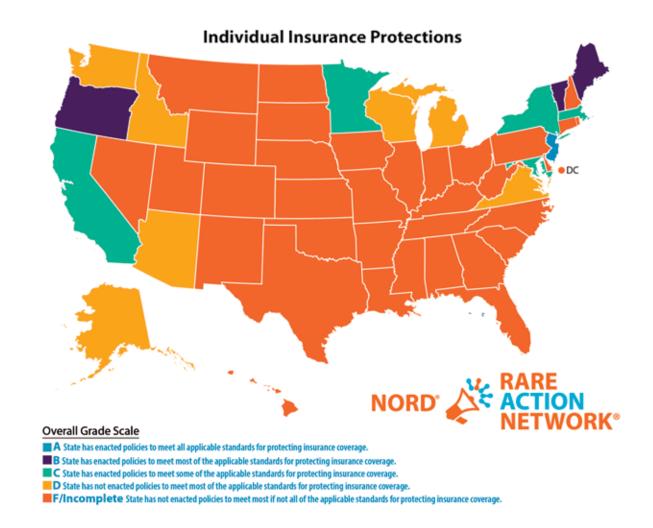
Given the fact that RDACs are a relatively new policy development in many states, NORD only graded states that have enacted an advisory council. For states that have not enacted an advisory council, grades are marked as "incomplete." In addition, many states have existing advisory structures that are 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 below details how NORD evaluated current and proposed advisory councils. The complete analysis is available in the appendix to this report.

#### Table 8: Rare Disease Advisory Councils Grading Rubric

	DESCRIPTION
GRADE	Rare Disease Advisory Councils Grading
A	State has a rare disease advisory council that is meeting statutory require- ments, including a majority of appointments
В	State has established a temporary rare disease task force or rare disease legislative caucus that is meeting statutory requirements (if applicable)
C	State has a rare disease advisory council that is meeting some, but not all, statutory requirements, including making required appointments
D	State has a rare disease advisory council that is not meeting statutory requirements
Incomplete	State does not have a formal body to address rare disease policy issues





In recent years, Congress and the Administration have taken various actions to destabilize private insurance markets across the country. In 2018, prior to the open enrollment period in the Affordable Care Act (ACA) marketplaces, the Trump Administration cut the open enrollment period in half and substantially reduced the resources for certified health insurance navigators and enrollment assistants. In April 2018, the Administration announced various changes to the marketplaces within its Notice of Benefit and Payment Parameters for 2019 final rule that allow states to weaken their essential health benefit and network adequacy requirements.<sup>12</sup> In December 2017, Congress lowered the penalty for violating the ACA individual mandate to zero as part of the Tax Cuts and Jobs Act, in effect repealing the mandate. In August 2018, the Administration released its Short-Term, Limited-Duration Insurance final rule, which allows for the expanded use of short-term, limited-duration health plans and association health plans, both of which include fewer comprehensive requirements for coverage and benefits. Finally, the Administration has declined to defend the constitutionality of the ACA in a lawsuit brought by some state attorneys general that, if successful, could mean the

<sup>&</sup>lt;sup>12</sup> "Patient Protection and Affordable Care Act; HHS Notice of Benefit and Payment Parameters for 2019." Federal Register. April 17, 2018. <u>https://www.federalregister.gov/documents/2018/04/17/2018-07355/patient-protection-and-affordable-care-act-hhs-notice-of-benefit-and-payment-parameters-for-2019.</u>



end of the ACA and the protections it affords all those with pre-existing conditions.

Each of these actions has contributed to the destabilization of private insurance markets within the states and has threatened access to adequate and affordable coverage for rare disease patients. However, there are various actions states can pursue to counter or mitigate these damaging actions. For example, some states have chosen to enact their own individual mandates and tightly regulate the sale of shortterm, limited-duration health plans and association health plans. Others have sought to implement reinsurance policies through a section 1332 waiver that help to stabilize the marketplace and keep costs low for all.

#### **GRADING METHODOLOGY**

NORD encourages states to take on efforts to stabilize and strengthen their health insurance markets in order to ensure individuals with rare diseases and their families can access adequate and affordable coverage. It is important to note, however, that this is a new and rapidly evolving area of policy. Consequently, lower grades in this category are not necessarily indicative of a state's intention or future policy goals. To reflect this dynamic, the "F" grade for this category is also listed as "incomplete." The grading rubric for Individual Insurance Protections can be found on pages 32-33. NORD graded the following three individual policy sections, weighted them equally and combined them to determine an overall grade:

- Pre-existing Conditions Protections: If the ACA is struck down in court, many patients with pre-existing conditions will once again be vulnerable to discrimination on the part of insurers. States can mitigate this challenge by creating their own pre-existing conditions protections. These include:
  - a. Guaranteed issue
  - b. Community rating
  - c. Elimination riders
  - d. Individual mandate

- Association Health Plans and Short-Term, Limited-Duration Health Plans: With the potential of short-term, limited-duration health plans and association health plans to divide the marketplace into people in need of more comprehensive health coverage and relatively healthy people who may not need extensive coverage, states ought to enact policies that prevent such segmentation. These include:
  - a. Limitations placed on association health plans
  - b. Limits on the initial contract duration of short-term, limited-duration health plans
  - c. Prohibition on renewability of short-term, limited-duration health plans
  - d. Limitations on the duration of renewability periods for short-term, limited-duration health plans
- 3. Reinsurance: NORD analyzed whether states have attempted to stabilize premiums by requesting and receiving a section 1332 waiver from the Centers for Medicare and Medicaid Services (CMS) to create a reinsurance program for particularly expensive beneficiaries. The components of our grading for these programs consisted of the following:
  - a. Whether the state sought to implement a reinsurance program through a section 1332 waiver
  - b. Whether the reinsurance program is attachment point or conditions-based (attachment point means the program applies to anything above a certain cost whereas conditions-based means the program applies only to specific conditions)



#### Table 9: Grading Rubric for State Pre-Existing Conditions Protections

	DESCRIPTION			
	Pre-existing Conditions Protections			
GRADE	Guaranteed Issue	Community Rating	Elimination Riders	Status of the State Individual Mandate
A	Insurers must issue all coverage plans to all individuals	Rating for health status prohibited	Elimination riders prohibited	Mandate in place
В	Insurers must issue all coverage plans to all individuals	Rating for health status prohibited	Elimination riders prohibited	No mandate
C	Insurers must issue to HIPAA eligible individuals OR the state has identified an insurer of last resort	Rating for health status not prohibited under state law	Elimination riders prohibited	No mandate
D	Insurers must issue to HIPAA eligible individuals OR the state has identified an insurer of last resort	Rating for health status not prohibited under state law	Elimination riders not prohibited under state law	No mandate
F/ Incomplete	Federal HIPAA protection only	Rating for health status not prohibited under state law	Elimination riders might not be prohibited under state law	No mandate

#### Table 10: Grading Rubric for Association Health Plans and Short-Term, Limited-Duration Health Plans

	DESCRIPTION				
	limitations placed on	Short-Term, Limited-Duration Health Plans			
GRADE	DE Limitations placed on Association Health Plans	Limit on initial contract duration	Are plans renewable	Limitations on renewability	
A	Yes	Short term plans not allowed in the state	No	Plans are not renewable	
В	Yes	<6 months or 185 days	No	Plans are not renewable	
C	State may place limitations on association health plans	<6 months or 185 days	No	Plans are not renewable	
D	State may place limitations on association health plans	<12 months or 365 days	Yes	State may have limitations on renewability	
F/ Incomplete	State may place limitations on associa- tion health plans	No limit	Yes	No limit	



#### **Table 11: Grading Rubric for Reinsurance**

	DESCRIPTION		
GRADE	Has the state obtained a 1332 waiver to establish a reinsurance program?	Is the reinsurance program attachment point or conditions based?	
A	Yes	Attachment point model or combination	
В	Yes	Conditions-based only	
C/D	Yes	Attachment point or conditions-based model, but waiver also has other program restrictions that limit availability to people with high-cost medical conditions	
F/ Incomplete	No	No reinsurance program	

### Resources

#### **MEDICAL NUTRITION**

- 1. National PKU Alliance, State Coverage Database. http://npkua.org/TakeAction/StateCoverage.aspx\_\_\_\_\_
- 2. Children's Magic U.S., Coverage States. http://childrensmagicus.org/coverage-states/
- American Partnership for Eosinophilic Disorders (Apfed), State Insurance Mandates for Elemental Formula. <u>http://apfed.org/advocacy/state-insurance-mandates-for-elemental-formula/</u>
- HRSA, State Statutes and Regulations on Dietary Treatment of Disorders Identified Through Newborn Screening. 2014. <u>http://www.hrsa.gov/advisorycommittees/mchbadvisory/ heritabledisorders/reportsrecommendations/reports/ statelaws.pdf</u>
- 5. Nutrica Metabolics, State Coverage Database. <u>http://www.medicalfood.com/Reimbursement/Coverage-by-State/</u>

#### **RX OOP**

- 6. NORD analysis of each state's existing statute and regulation. NORD analysis of legislative tracking information obtained through the State Access to Innovative Medicines (SAIM) coalition.
- State Patients Equal Access Coalition (SPEAC). National Landscape of Caps on Patient Out-of-Pocket & State Oral Chemotherapy Parity Laws.

#### **NEWBORN SCREENING**

- 8. NORD analysis of state statute and regulation.
- HRSA, Advisory Committee on Heritable Disorders in Newborns and Children. Recommended Uniform Screening Panel. <u>http://www.hrsa.gov/advisorycommittees/mchbadvisory/ heritabledisorders/recommendedpanel/</u>
- 10. Baby's First Test, Conditions Covered by State. <u>http://www.babysfirsttest.org/newborn-screening/states</u>
- 11. National Newborn Screening and Genetics Resource Center (NNSGRC), National Newborn Screening Status Report. <u>http://genes-r-us.uthscsa.edu</u>
- 12. Association of Public Health Laboratories (APHL), NewSTEPS Screened Conditions Report. <u>https://data.newsteps.org/newsteps-web/reports/</u> <u>screenedConditions/list.action</u>
- 13. APHL, NewSTEPS Data Repository State Profiles. <u>https://data.newsteps.org/newsteps-web/stateProfile/</u> <u>input.action</u>

#### MEDICAID

- 14. NORD analysis of proposed 1115 waivers. https://www.medicaid.gov/medicaid/section-1115-demo/ index.html
- 15. Kaiser Family Foundation State Health Facts, Status of State Action on the Medicaid Expansion Decision. <u>https://www.medicaid.gov/medicaid/section-1115-demo/</u> <u>index.html</u>
- 16. NCSL, Medicaid and CHIP Eligibility by State. <u>http://www.ncsl.org/research/health/medicaid-eligibility-</u> <u>table-by-state-state-activit.aspx</u>

### Resources

#### MEDICAID HOME AND COMMUNITY-BASED SERVICES WAIVERS

- 17. NORD analysis of HCBS waivers and state plans. https://www.medicaid.gov/medicaid/ltss/index.html.
- Kaiser Family Foundation State Health Facts, Medicaid Home and Community Based Services: Results from a 50-State Survey of Enrollment, Spending, and Program Policies. <u>https://www.kff.org/medicaid/report/medicaid-home-andcommunity-based-services-results-from-a-50-state-surveyof-enrollment-spending-and-program-policies/</u>

#### **STEP THERAPY**

- 19. NORD analysis of state statute and regulation.
- 20. National Psoriasis Foundation analysis of step therapy implementation in the states. https://www.psoriasis.org/advance/step-therapy-states

#### **RARE DISEASE ADVISORY COUNCILS**

21. NORD analysis of state statutes.

#### INDIVIDUAL INSURANCE PROTECTIONS

- 22. NORD analysis of state statutes and regulation.
- 23. Kaiser Family Foundation State Health Facts, Individual Market Rate Restrictions. <u>https://www.kff.org/other/state-indicator/individual-mar-ket-rate-restrictions-not-applicable-to-hipaa-eligible-individuals/?currentTimeframe=0&sortModel=%7B%22colld%22 :%22Location%22,%22sort%22:%22asc%22%7D</u>
- 24. Health Insurance and Individual States Overview. National Conference of State Legislatures. <u>http://www.ncsl.org/research/health/health-insurance-and-states-overview.aspx.</u>
- 25. In the Wake of New Association Health Plan Standards, States Are Exercising Authority to Protect Consumers, Providers, and Markets. Commonwealth Fund. <u>https://www.commonwealthfund.org/blog/2018/initialstate-approaches-association-health-plans. Accessed</u> <u>March 27, 2019.</u>

### **SPECIAL THANKS**

# NORD would like to thank the following people and organizations for their contributions to this report:

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State Access to Innovative Medicines (SAIM) Coalition



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