NORD: The Independent Voice of the Rare Disease Patient Community

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

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

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

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

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

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

NORD Mission Statement

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

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

1. Medical foods coverage requirements for commercial health plans
2. Medical foods coverage requirements for state-run programs
3. Capitation of prescription drug cost sharing in commercial health plans
4. Adoption of federally-recommended (RUSP) newborn screening Core conditions
5. Adoption of RUSP newborn screening Secondary conditions
6. State procedures for adding new diseases to its newborn screening panel
7. Rules on the storage and research uses of newborn screening dried blood spots
8. Medicaid eligibility levels, including for the Children’s Health Insurance Program (CHIP)
9. Medicaid 1115 waivers for work requirements and formulary exclusions
10. Biosimilar prescriber communication
11. Patient protections against Step Therapy (fail first) protocols
12. Establishment of Rare Disease Advisory Councils
13. Right to Try requirements

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

National Overview

Medical Foods: State-Run Coverage

National Overview

Medical Foods: State-Run Coverage

Overall Grade Scale
- A: Coverage mandated with minimal restrictions on age/dollar value.
- B: Coverage mandated but with restrictions on age or dollar value, minimal restrictions on covered disorders.
- C: Some coverage restrictions based on age/dollar value and covered disorders.
- D: Severe coverage restrictions and excludes many rare disorders.
- F: No mandated coverage.

Medical Foods: Private Insurance Coverage Requirements

Overall Grade Scale
- A: Coverage mandated with minimal restrictions on age/dollar value.
- B: Coverage mandated but with restrictions on age or dollar value.
- C: Some coverage restrictions based on age/dollar value and covered disorders.
- D: Severe coverage restrictions and excludes many rare disorders.
- F: No mandated coverage.

National Organization for Rare Disorders: 2018 State Action Report

National Overview

Medical Foods: State-Run Coverage

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- C: Some coverage restrictions based on age/dollar value and covered disorders.
- D: Severe coverage restrictions and excludes many rare disorders.
- F: No mandated coverage.

National Organization for Rare Disorders: 2018 State Action Report
Newborn Screening: RUSP Core Conditions

Overall Grade Scale

- A: State screens for all RUSP recommended conditions
- B: State screens for at least 75% of RUSP recommended conditions
- C: State screens for at least 50% of RUSP recommended conditions
- D: State screens for at least 25% of RUSP recommended conditions
- F: No policy

Newborn Screening: RUSP Secondary Conditions

Overall Grade Scale

- A: State detects all RUSP Secondary recommended conditions
- B: State detects at least 75% of RUSP Secondary recommended conditions
- C: State detects at least 50% of RUSP Secondary recommended conditions
- D: State detects at least 25% of RUSP Secondary recommended conditions
- F: State requires detection of more than 15 Secondary recommended conditions

Adding New Conditions to NBS Panels

Overall Grade Scale

- A: Only DOH/Advisory Committee approval required for new conditions. State follows the RUSP but no timeline
- B: Only DOH/Advisory Committee approval required. State follows the RUSP but no timeline
- C: Only DOH/Advisory Committee approval required for new conditions. State requires pilot testing but no timeline for following the RUSP
- D: Legislative approval required
- F: No policy

Rx OOP Protections

Overall Grade Scale

- A: Rx OOP protections apply for all prescription drugs that apply pre-deductible
- B: Rx OOP protections apply for up to 3 RUSP conditions
- C: Rx OOP protections do not apply pre-deductible and may not cover all costs
- D: Rx OOP protections do not apply for more than 5 RUSP conditions
- F: No policy

National Overview (continued)
Dried Blood Spot Storage and Research Uses

Overall Grade Scale

- **A**: DBS consented for research and stored for 10 years or longer
- **B**: DBS consented for research and stored for up to 10 years
- **C**: DBS consented for research and stored up to 5 years
- **D**: DBS consented for research but only stored for up to 6 months
- **F**: DBS not consented for research

Medicaid Eligibility for Childless Adults

Overall Grade Scale

- **A**: Eligibility at 138% of the Federal Poverty Level (FPL) or greater
- **B**: Eligibility at 120% to 137% of FPL
- **C**: Eligibility at 90% to 137% of FPL
- **D**: Eligibility at 89% of FPL or less
- **F**: No eligibility for Childless Adults

Medicaid Waivers

Overall Grade Scale

- **A**: State has proposed or adopted a waiver that would limit eligibility or benefits
- **B**: State has no policy in place

Biosimilar Prescriber Communication

Overall Grade Scale

- **A**: State requires prescriber notification and allows physician override
- **B**: State requires prescriber notification but no physician override
- **C**: Policy allows prescriber communication but does not mandate it
- **D**: N/A
- **F**: No policy in place

National Organization for Rare Disorders: 2018 State Action Report

National Overview (continued)
National Overview (continued)

Step Therapy (Fail First)

Overall Grade Scale:
- A: State has protections that allow physician override
- B: State has protections but no physician override
- C: Incomplete
- D: State only requires patient to be notified of step therapy
- F: No protections in place

Right to Try

Overall Grade Scale:
- State has no policy in place
- State has passed Right to Try law (that NORD does not support)
- State has no policy in place

Rare Disease Advisory Councils

Overall Grade Scale:
- A: Permanent Rare Disease Advisory Council
- B: Temporary council or Task Force
- C: Rare disease legislative caucus
- Incomplete

[Map images showing the states' grades for step therapy and right to try laws]
SECTION II
EXPLORING THE ISSUES

STATE COVERAGE REQUIREMENTS FOR COMMERCIAL HEALTH PLANS

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

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

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

Because insurance is regulated primarily at the state level, many states have mandated the inclusion of medical foods within private plans sold within their state. However, in the states that do not have medical food mandates, individuals in need of these particular treatments are faced with a huge burden of access and require assistance in paying for medical food expenses.
Coverage of medical foods within each state’s Medicaid program is also essential, yet only some states mandate coverage. For states that do mandate coverage through Medicaid, a few have chosen to provide access to medical foods through other publicly-funded health programs or provide coverage on a case-by-case basis (which can lead to high variability in which patients have access).

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

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

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

### Methodology

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

### Covered Disorders

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

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### Table 1: Medical Foods Grading Rubric

<table>
<thead>
<tr>
<th>Grade</th>
<th>Private Insurance Coverage Requirements (Including Covered Disorders)</th>
<th>State-Run Coverage</th>
</tr>
</thead>
<tbody>
<tr>
<td>A</td>
<td>Coverage is required for both formula and low-protein foods with no limits on eligibility or coverage. Covered disorders include all inborn errors of metabolism; eosinophilic disorders/FPIES; or medically necessary treatment.</td>
<td>Mandated Medicaid coverage for medical foods with no age or eligibility restrictions (or through a supplemental program). Covered disorders include all inborn errors of metabolism; eosinophilic disorders/FPIES; or medically necessary treatment.</td>
</tr>
<tr>
<td>B</td>
<td>Coverage is required for formula and low-protein food but with age or dollar limits. Covered disorders include all inborn errors of metabolism; eosinophilic disorders/FPIES; or medically necessary treatment.</td>
<td>Mandated Medicaid coverage for formula and low-protein foods (or through a supplemental program) with restrictions. Covered disorders include all inborn errors of metabolism; eosinophilic disorders/FPIES; or medically necessary treatment.</td>
</tr>
<tr>
<td>C</td>
<td>Coverage is required for both formula and low-protein foods, but with age and dollar limits. Covered disorders do not include eosinophilic disorders, FPIES, and other medically necessary uses.</td>
<td>Coverage for formula and low-protein foods is on a case-by-case basis. Covered disorders do not include eosinophilic disorders, FPIES, and other medically necessary uses.</td>
</tr>
<tr>
<td>D</td>
<td>Coverage required but with limits on eligibility (such as age) or coverage (such as a dollar cap or formula only). Covered disorders include 3 or fewer metabolic conditions (such as PKU-only) and do not include eosinophilic disorders, FPIES, and other medically necessary uses.</td>
<td>Mandated Medicaid coverage for formula but no coverage of low-protein foods. Covered disorders include two or fewer metabolic conditions (such as PKU-only) and do not include eosinophilic disorders, FPIES, and other medically necessary uses.</td>
</tr>
<tr>
<td>F</td>
<td>State does not mandate private insurance coverage of medical foods.</td>
<td>State does not mandate coverage for Medicaid. The state does not offer supplemental programs to provide coverage.</td>
</tr>
</tbody>
</table>
many people with rare disorders, as well as those with other severe chronic diseases, these costs are untenable. As a consequence, patients in need of life saving treatment are forced to go without their medication or use options that are less effective and less safe.

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

To assist patients who find themselves in this difficult situation, several states have passed legislation mandating a limit on out-of-pocket costs for specialty medications. These limits range from $100 to $500 per month per-medication, depending on the type of plan. NORD strongly supports the enactment of these types of policies as they greatly benefit rare patients at a minimal impact to the overall insured population. In fact, third-party analysis has demonstrated that these types of limits on co-pays can be instituted with little to no impact on overall plan premiums for all beneficiaries.1

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

This proposal is also highly feasible to implement. Legislation based on a “copay-only” rule has already been adopted by Colorado’s insurance commissioner in 2015.1 That rule required insurers to offer at least some plans that feature more affordable drug coverage. In that state, patients now have different types of plans to choose from, including some that use only co-pays in the drug benefit, and they do not have to pay significantly higher premiums for one of these copay-only plans than for other kinds of plans.

### METHODOLOGY

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

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

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

<table>
<thead>
<tr>
<th>GRADE</th>
<th>Rx Cost Sharing Grading Rubric</th>
</tr>
</thead>
<tbody>
<tr>
<td>A</td>
<td>State has instituted a total cap or per-drug cap on Rx cost sharing that applies pre-deductible and for all prescription drugs.</td>
</tr>
<tr>
<td>B</td>
<td>State has instituted a per-drug cap or total cap on Rx cost sharing that applies pre-deductible. Cap only applies to specialty-tier drugs. OR State requires 25% of eligible plans to offer a copay-only benefit for prescription drugs.</td>
</tr>
<tr>
<td>C</td>
<td>State has instituted a per-drug cap or total cap on cost sharing that does not apply pre-deductible.</td>
</tr>
<tr>
<td>D</td>
<td>State has enacted cost sharing limits for a limited number of treatments (such as oral chemotherapy only).</td>
</tr>
<tr>
<td>E</td>
<td>State does not have a cap on cost sharing.</td>
</tr>
</tbody>
</table>
Newborn screening (NBS) is one of the most successful public health programs ever enacted, saving thousands of lives over the past 50 years. Newborn screening allows physicians to catch a heritable disease early and start treatment almost immediately following birth. In this way, many of the worst effects of a disease can be mitigated.

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

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

BACKGROUND

Although newborn screening (NBS) is one of the most successful public health programs ever enacted, state laws and regulations for newborn screening programs vary. As a result, newborn screening programs can have great leeway in terms of what conditions to screen for and how samples are used following a blood spot test.

STATES HAVE GREAT LEWAY

Almost entirely at the state level, newborn screening programs can have great leeway in terms of what conditions to screen for and how samples are used following a blood spot test.

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

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

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

- **DRIED BLOOD SPOT RESEARCH USES**: We analyzed how long states allow DBS to be stored and whether they allow research uses (with parent consent).

- **STATE PROCESS FOR ADDING NEW CONDITIONS**: We analyzed the process by which states can add new conditions to its NBS panel (and whether every condition is mandated for all populations). This report has expanded its analysis of NBS to better capture these issues and compare state programs.

**STORAGE AND RESEARCH USES OF DRIED BLOOD SPOTS USED IN NBS**

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

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

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

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

<table>
<thead>
<tr>
<th>Table 3: Newborn Screening Grading Rubric</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>GRADE</strong></td>
</tr>
<tr>
<td>A</td>
</tr>
<tr>
<td>B</td>
</tr>
<tr>
<td>C</td>
</tr>
<tr>
<td>D</td>
</tr>
<tr>
<td>F</td>
</tr>
</tbody>
</table>

Table 4: Newborn Screening: RUSP Core Conditions

For more information on the diseases listed, visit NORD’s Rare Disease Database or the National Institutes of Health.

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

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

<table>
<thead>
<tr>
<th>Condition</th>
<th>Description</th>
</tr>
</thead>
</table>
| CACT            | Carnitine-acylcarnitine translo-
|                 | case 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                 |
| GA 2            | Glutaric academia type II             |
| MCKAT           | Medium-chain leucyl-CoA thiolase      |
|                 | deficiency                            |
| M/SCHAD         | 3-Hydroxyacyl-CoA dehydrogenase       |
|                 | deficiency                            |
| SCAD            | Short-chain acyl-CoA dehydroge-
|                 | nase deficiency                       |
| 2M3HBA          | 2-Methyl-3-hydroxybutyric academia    |
| 2MBG            | 2-Methylbutyryl-CoA dehydroge-
|                 | nase deficiency                       |
| 3MGA            | 3-Methylglutaconyl-CoA hydra-
|                 | tase deficiency                       |
| CH-C,D          | Cobalamin C cofactor deficiency      |
| IBG             | Isobutyrylglycinuria                  |
| MAL             | Mal de Meleda                         |
| ARG             | Argininemia                           |
| BIODPT-BS       | Bioppterin defect in cofactor       |
|                 | biosynthesis                          |
| BIODPT-RG       | Bioppterin defect in cofactor       |
|                 | regeneration                          |
| CTF-II          | Citrullinemia, Type II                |
| H-PHE           | Hyperphenylalaninemia                 |
| MET             | Hypermethioninemia                    |
| TYR-II          | Tyrosinemia, Type II                 |
| TYR-III         | Tyrosinemia, Type III                |
| GALC            | Galactoepimerase deficiency          |
| GALK            | Galactokinase deficiency             |
| HBS             | HBS disease                          |

Table 5: Newborn Screening Secondary Conditions

Medicaid

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

The State Children’s Health Insurance Program (CHIP) is an important source of health coverage for children and families that are ineligible for traditional Medicaid. All states provide increased coverage for children and families through CHIP but may operate the program slightly differently. For example, some states use the federal funding for CHIP to expand their Medicaid program to reach this target population (this is sometimes referred to as “CHIP-funded eligibility”). Other states use these funds to operate a separate CHIP program that provides separate coverage from their Medicaid program.
BACKGROUND ON MEDICAID 1115 WAIVERS

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

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

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

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

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

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

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

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

METHODOLOGY

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

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

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

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

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

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

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

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

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

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

METHODOLOGY

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

### Table 7: Biosimilars Grading Rubric

<table>
<thead>
<tr>
<th>GRADE</th>
<th>DESCRIPTION</th>
</tr>
</thead>
<tbody>
<tr>
<td>A</td>
<td>Policy requires pharmacist to notify prescriber before making a substitution. Policy allows physician to override biosimilar substitution</td>
</tr>
<tr>
<td>B</td>
<td>Policy requires communication but does not allow for physician override</td>
</tr>
<tr>
<td>C</td>
<td>Policy includes prescriber communication but does not mandate it</td>
</tr>
<tr>
<td>D</td>
<td>--</td>
</tr>
<tr>
<td>F</td>
<td>No policy</td>
</tr>
</tbody>
</table>

### BACKGROUND

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

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

NORD supports state efforts to place adequate patient protections around the use of step therapy that will ensure...
patients are protected. The main features of these protections are as follows:

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

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

**METHODOLOGY**

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

### Table 8: Step Therapy Grading Rubric

<table>
<thead>
<tr>
<th>GRADE</th>
<th>DESCRIPTION</th>
</tr>
</thead>
<tbody>
<tr>
<td>A</td>
<td>State requires step therapy to be based on independent medical criteria. State requires plans to establish a clear exceptions process for patients and providers. State allows prescribing physician to override step therapy based on medical criteria.</td>
</tr>
<tr>
<td>B</td>
<td>State requires step therapy to be based on independent medical criteria. State requires plans to establish a clear exceptions process for patients and providers.</td>
</tr>
<tr>
<td>C</td>
<td>State only requires patients to be notified of the implementation of step therapy.</td>
</tr>
<tr>
<td>D</td>
<td>State only requires patients to be notified of the implementation of step therapy.</td>
</tr>
<tr>
<td>F</td>
<td>No policy</td>
</tr>
</tbody>
</table>

**BACKGROUND**

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

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

Ultimately, NORD believes that the establishment of a focused rare disease advisory council can help pave the way for better health care policy in a given state, therefore, we are strongly supporting the work of local advocates to create new councils in their state.
To date, states with existing Rare Disease Advisory Councils (such as Pennsylvania and North Carolina) have shown that this model is an effective way to increase government awareness on issues like newborn screening and Medicaid restrictions for new orphan drug approvals.

**METHODOLOGY**

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

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

**Table 9: Rare Disease Advisory Councils Grading Rubric**

<table>
<thead>
<tr>
<th>GRADE</th>
<th>DESCRIPTION</th>
</tr>
</thead>
<tbody>
<tr>
<td>A</td>
<td>Permanent Rare Disease Advisory Council</td>
</tr>
<tr>
<td>B</td>
<td>State established temporary Rare Disease Task Force</td>
</tr>
<tr>
<td>C</td>
<td>Rare Disease Legislative Caucus</td>
</tr>
<tr>
<td>D</td>
<td>No formal body to address rare disease policy issues</td>
</tr>
<tr>
<td>F</td>
<td>No formal body to address rare disease policy issues</td>
</tr>
</tbody>
</table>

**BACKGROUND**

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

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

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

Dr. Scott Gottlieb, FDA Commissioner:

"I think there is a perception, ...that there are certain
Dr. Ellen Sigal, Chair of Friends of Cancer Research:

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

Mr. Kenneth Moch, President and CEO of Cognition Therapeutics:

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

Right to Try legislation is going to make more people have

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

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

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

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

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

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

EMS PROTOCOLS FOR RARE DISEASES

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

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

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

TELEMEDICINE

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

PRIVATE INSURANCE MARKET STABILIZATION

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

Prior to the 2018 open enrollment period in the Affordable Care Act (ACA)
marketplaces, the Trump Administration cut the open enrollment period in half, and substantially reduced
the resources for certified health insurance navigators and enrollment assistants. In November, the Administration proposed various changes to the marketplaces within their Notice of Benefit and Payment Parameters Proposed Rule that could allow states to weaken their essential health benefits and network adequacy requirements. In December, Congress repealed the ACA individual mandate as part of the Tax Cuts and Jobs Act. The Trump Administration is also proposing to expand the use of association health plans and short-term plans, both of which include more relaxed requirements for coverage and benefits.

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

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

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

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

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

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

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

**LOOKING FORWARD**

**RARE ACTION NETWORK™**

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

RAN is the nation’s leading rare disease advocacy network working to improve the lives of the 30 million Americans impacted by rare disease. The goal of RAN is to ensure that the rare disease community is represented and supported in all 50 states. RAN serves as a broad spectrum of stakeholders ranging from patients, to their families, caregivers, and friends; from researchers to industry; to physicians and academia. While working on both the national and state level, RAN filters information to help address issues of national concern and engage rare communities to take action through policy, awareness, and education initiatives in their state and local communities.

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

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

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

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

**JOIN THE NETWORK**

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

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

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

**JOIN THE NETWORK**

National Organization for Rare Disorders: 2018 State Action Report
Prescription Drug Cost Sharing


2. Avalere PlanScape®, a proprietary analysis of exchange plan features, shared by SAIM members.

3. NORD identified state legislation and regulation through a state by state legislative analysis. As part of the State Access to Innovative Medicines (SAIM) coalition, NORD also had access to legislative tracking information and legislative analysis. As part of the State Access to Innovative Medicines (SAIM) coalition, NORD also had access to legislative tracking information and legislative analysis. As part of the State Access to Innovative Medicines (SAIM) coalition, NORD also had access to legislative tracking information and legislative analysis.


Newborn Screening


4. Newborn Screening and Genetics Resource Center (NNSGRC), National Newborn Screening and Genetics Resource Center, https://www.nngrc.org/docs/medical-foods/NutriciaMetabolics,


Medicaid

1. https://www.cms.gov/Medicare/Prescription-Drug-Coverage/PartD/PartDPrescriber-Education.html


Biosimilars


2. NORD analysis of 2017 state legislation

Step Therapy


2. NORD analysis of state legislation


Right to Try


2. FDA. “FDA Has Taken Steps to Improve the Expanded Access Program but Should Further Clarify How Adverse Events Data Are Used,” July 2017.


4. Ibid.

5. NORD analysis of state legislation


Sources by Topic

Medical Food Sources


6. NORD ID-126

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