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Henry McMaster, Governor of South Carolina
Harvey S. Peeler, Jr, Chairman, Senate Finance Committee
Daniel B. Verdin, III, Chairman, Senate Medical Affairs Committee
Bruce W. Bannister, Chairman, House Ways and Means Committee
Leon Howard, Chairman, House Medical, Military, Public and Municipal Affairs Committee

June 26, 2024

Re: Progress report for the SC Rare Diseases Advisory Council

Dear Governor and respective Chairmen,

On behalf of the members of the South Carolina Rare Diseases Advisory Council, it is my pleasure to submit the second annual legislative report as outlined in Section 44-1-320 of Bill H.3956. This report documents the key initiatives related to research, advocacy, and awareness that the council sought to achieve over the past year. Please do not hesitate to reach out with any questions or comments.

Sincerely,

Patrick A Flume, M.D.

The Powers-Huggins Endowed Chair for Cystic Fibrosis
Distinguished Professor of Medicine and Pediatrics
Chair, SC Rare Disease Advisory Council
Director, MUSC Cystic Fibrosis Center
Associate Vice President for Clinical Research, MUSC

Executive Summary

Since the last progress report submitted in June 2023, the council has tackled several key initiatives as outlined in their official responsibilities. Main accomplishments of the council during the 2023-2024 year are highlighted below:

- Hiring a Program Coordinator to assist with promoting council objectives;
- Holding quarterly meetings open to the general public to encourage statewide collaboration on, and support of, council initiatives;
- Hosting an inaugural Rare Disease Symposium to promote research and spread awareness of and advocacy for the state's rare disease community;
- Revamping the official website (rarediseasesc.org) to optimize awareness of state resources and foster public engagement; and
- Compiling and disseminating a needs assessment survey for the state's rare disease community.

The council relied upon heavy media campaigning to promote both the inaugural symposium and the needs assessment survey. The digital flyer promoting the symposium and the program agenda are included in Appendix A for review. Access to the survey can be found on the home page of rarediseasesc.org.

The council reviewed the State Report Card as evaluated by the National Organization of Rare Diseases (NORD). Based on their assessment South Carolina scores poorly in most areas and that is provided in Appendix B. NORD also provided recommendations as to how the state could improve its assessment. The council intends to further review these recommendations in order to offer recommendations to the legislature as well as suggest priorities.

The council's mission, membership, and official responsibilities are provided to restate the council's purpose and role as a statewide project. The minutes from the quarterly meetings of the SC RDAC and the expense report follow in this progress report.

South Carolina Rare Diseases Advisory Council

Mission

The council shall advise the Governor, the General Assembly, and other stakeholders on research, diagnosis, treatment, and education relating to rare diseases.

Membership

The council is composed of fifteen members and intended to be broadly representative of stakeholders. Each position is appointed by statewide leadership including the President of the Medical University of South Carolina (MUSC), the Dean of the University of South Carolina (UofSC) School of Medicine, the Director of the Department of Health and Environmental Control (DHEC), the Director of the Department of Health and Human Services (DHHS), the Executive Director of the South Carolina Hospital Association (SCHA), the Executive Director of the South Carolina Primary Healthcare Association (SCPHA), and the Executive Director of the State Public Benefit Authority (SPBA).

| Representation | Member |
|---|---|
| Chair | Patrick Flume, MD (MUSC) |
| Department of Health and Environmental Control | Ginie Daguise |
| Department of Health and Human Services | Kevin Wessinger |
| Medical University of South Carolina | Dan Judge, MD (MUSC, cardiology) |
| University of South Carolina School of Medicine | Divya Ahuja, MD (Infectious Diseases) |
| South Carolina Hospital Association | Kate Wink (Santee Cooper) |
| South Carolina Primary Healthcare Association | Vicki Young (SCPHCA) |
| Biopharma Industry | Jonathan Hawayek (SPARK Therapeutics) |
| Research and Treatment of Rare Diseases (3) | Maysen Mesaros, MS (genetic counselor) Neena Champaigne, MD (pediatric genetics) Chip Norris, PhD (MUSC, connective tissue) |
| Patient (2) | Anthony Hamilton |

| | |
|---------------------------------------|---|
| | Karen Kemper, PhD |
| Rare Disease Organization | Megan Onysko stepped down; search ongoing for new membership |
| Caregiver of person with rare disease | Jason Smith |
| State Health Plan | Tripp Jennings, MD (VP and Clinical Innovations Officer, BCBS South Carolina) |
| Program Coordinator | Kendall Spillman |

Responsibilities of the Advisory Council

- Solicit comments from stakeholders, including patients and patient caregivers in South Carolina impacted by rare diseases, to assess the needs of rare-disease patients, caregivers, and providers in the State.
- Consult with experts on rare diseases to develop recommendations to improve patient access to and quality of rare-disease specialists, affordable and comprehensive health care coverage, relevant diagnostics, timely treatment, and other needed services.
- Research and identify priorities related to treatments and services provided to persons with rare diseases in South Carolina and develop recommendations that include safeguards against discrimination for these populations on such issues, including disaster and public health emergency-related planning.
- Publish a list of existing, publicly accessible resources on research, diagnosis, treatment, and education relating to the rare diseases in South Carolina.
- Identify and distribute educational resources to foster recognition and optimize treatment of rare diseases in South Carolina.
- Identify best practices to reduce health disparities and achieve health equity in the research, diagnosis, and treatment of rare diseases in South Carolina.
- Report annually (by June 30) to the Governor, the Chairman of the Senate Finance Committee, the Chairman of the Senate Medical Affairs Committee, the Chairman of the House Ways and Means Committee, and the Chairman of the House Medical, Military, Public and Municipal Affairs Committee.

South Carolina Rare Diseases Advisory Council
Annual Report
June 26, 2024

Progress in the Last Year:

- A. Formation of a Program Coordinator: To meet the initiatives of the statewide project as outlined in the Proviso, the council saw fit to hire a Program Coordinator.
- B. Rare Disease Day Symposium:
 - a. The council held its inaugural Rare Disease Symposium on Leap Day, February 29, 2024, to coincide with annual Rare Disease Day. The purpose of the symposium was to promote research, advocacy, and awareness for patients with rare diseases in our state. The catered hybrid event was held at Segra Park in Columbia, SC from 10 am-2 pm, and had 47 online attendees and 26 in-person attendees.
 - b. Following the symposium, an evaluation feedback survey was sent to the attendees. 17 recorded respondents provided feedback. The majority stated that they found patient outreach and engagement, as well as education on advocacy, to be the most valuable aspects of the symposium.
- C. Needs Assessment Survey: In late January, the council disseminated a needs assessment survey for patients and caregivers of patients with rare diseases in the state. As of now, the survey has approximately 40 valid respondents. The survey is anonymous and is not used for research purposes. It currently is available to the public. The council will find additional ways next year to promote the survey. A data analysis will be presented when enough sufficient respondent data has been gathered.
- D. NORD State Report Card 2024: The council invited NORD Policy Manager Carolyn Sheridan to give a presentation on SC's NORD State Report Card at their April meeting. As SC legislation determines financial and health care policies that impact the rare disease community, and subsequently NORD report card evaluations, the council intends to review other states' policies, determine where to prioritize our efforts, and provide feedback to the SC legislature to improve scores.
- E. A public website (rarediseasesc.org) has been further developed to include the profiles and work of the council (biographies, headshots, annual reports, meeting notices and minutes), resources available for patients and families, and news and patient stories at the national and state level from multimedia sources (online articles, podcasts, interviews, and videos). There is invitation for users to provide comments and questions to the council.
- F. The council convenes at quarterly meetings each year, coordinated by and recorded by the Program Coordinator. The minutes of these meetings are attached as an appendix and are available on the website.

Expense Report

The total estimated expenses for the SC RDAC grant up to 6/30/24 consisted of \$150,502. They were allocated to the following categories:

- Salaries: \$96,804
- Fringe: \$43,174
- Symposium and Outreach (including catering): \$9,269
- Program Coordinator Travel: \$905.

Meeting Minutes: 08/18/23

Meeting agenda:

1. Welcome
2. Changes to the committee: None
3. Reviewed minutes from previous meeting
4. Legislature approved funding of the RDAC in the proviso:
 - a. Funds will be transferred to MUHA
 - b. PF reached out to MUHA to learn about how to use those funds
5. Needs assessment survey.
 - a. Has been built into REDCap.
 - b. Is it possible to translate into other languages (e.g. Spanish)?
 - c. Implementation plans (committee to add to the list)
 - i. Greenwood Genetics (NC)
 - ii. State newborn screening program (NC)
 - iii. Will talk to NORD and create a link on the front page (Do I have a rare disease?)
 - iv. Links on the website (QR code)
 - v. Mobile clinics
 - vi. Advocacy groups
 - vii. Nonprofits (SCtogether)
 - viii. Libraries
 - ix. Can we talk to newspapers? (PF will talk to public relations)
 - x. Can we get a postcard to distribute? Digital postcard?
6. Review of state data:
 - a. Jon Hawayek previously provided list of 50 most prevalent conditions from SPARK.
 - b. Jon is still awaiting approval from Florida RDAC to get their research variables; they have not had a full meeting to discuss
 - c. Plan was to compare the state Medicaid database to determine the state prevalence for these conditions
 - i. This could inform strategy of building out resources list.
 - ii. PF will reach out to CA to see what has been done, she could run an analysis to get counts and drug utilization, perhaps by county. TJ will look into potential requirements for us seeing data summaries
7. Additional topics (still open):
 - a. NORD Annual Breakthrough Summit, October 15, Washington DC (Carolyn Sheridan)
 - i. 2023 NORD Summit website <https://nordsummit.org/>
 - b. Concern expressed about the context of “Rare” and how we might be able to communicate the message to legislators
 - c. Kevin Wessinger (pediatrician, CMO for DHHS) has experience with federal advocacy through the AAP – consider scheduling some one-on-one meetings with selected legislators with specific talking points (one pager)
 - d. Could set up legislative breakfast (modest expense)-aim for a date when a lot will be there
 - e. Advocacy items to be considered for website: emergency preparedness

8. Next meeting last quarter 2023

Next steps:

1. Send minutes to the committee
2. Will review Colorado survey and see if we want to make any changes and then finalize REDCap needs assessment survey and get it posted
3. Will get list of variables from Florida to try to search the SC databases on Top 50 conditions, CA will do the analysis, and look at other opportunities

Meeting Minutes: 01/05/24

Meeting agenda:

1. Patrick Flume: Welcome & reminder of state funding approval
 - a. Contract between MUSC and MUHA finalized; funding fairly restrictive to what we can do.
 - b. Allocated \$250,000 to use; cannot pay ourselves and likely cannot pay for travel.
2. Introductions
 - a. Kendall Spillman introduced as new RDAC Program Coordinator
3. Rare Disease Day Symposium
 - a. Venue- Segra Park Club Lounge February 29, 2024, 9:30 am-2 pm (1640 Freed Street, Columbia, SC 29201)
 - i. This is inaugural event; held on last day of February, this year being Leap Day, in conjunction with National Rare Disease Day
 - ii. Venue contract will include A/V support and catered boxed lunch
 - b. Attendees
 - i. Council feels this audience will likely be members of the public, more so than the scientific community
 1. Should still be scholarly enough for scientists to recognize potential interest areas or collaborations
 - ii. Event will be free and hybrid with livestream for public access
 1. Registration will be encouraged. Registration link to be forwarded to council when ready
 2. Council generally in favor of having event recorded. Will look into speaker consent for, and audience acknowledgement of, recording.
 - iii. Invitations for legislature
 1. They'll be in session on weekday, so might not be able to attend
 2. Last year's event was geared towards them, so it should be fine to have a different focus
 - c. Agenda
 - i. Divided between research and advocacy (2 hours for each)
 1. Initially thinking each speaker will be given 20 minutes to present, with 10-minute Q&A session, but this is not fixed
 2. Must be non-MUSC centric. Should include speakers from across the state.
 3. Council in agreement that a patient panel can be impactful for the public
 - ii. Potential agenda theme and outline:
 1. General Info on Clinical Trials & Patient Engagement
 - a. Presented by Dr. Flume?
 - b. Discuss the value of clinical trial participation, overcoming concerns related to genetic research, resources for getting involved in research (ClinicalTrials.gov, SCresearch.org, Researchmatch.org), etc.

2. Innovation and Discovery happening within South Carolina
 - a. Genetics and Rare Disease
 - i. Dr. Judge to discuss In Our DNA?
 - ii. DHEC overview of state-wide genetics testing efforts?
 - iii. Perhaps someone from Clemson to discuss their genetics program?
 - iv. Newborn Screening to be discussed by Neena Chapaigne
 1. Data to be shared to aid in discussion from SCDHEC?
 - v. Someone from Greenwood could walk through animal models / functional studies?
 - b. Diseases focused presentation?
 - i. Chip Norris to present on Ehlers Danlos Syndrome research
3. Patient Experience and Advocacy
 - a. Consider inviting patients to speak who also have a role in or experience with advocacy work, so they can speak to both personal experience and advocacy efforts (3-4 person panel)
- d. Evaluation
 - i. Council to debrief post-symposium
 - ii. Post-event survey to be distributed to attendees for feedback
4. Other council projects
 - a. Needs Assessment survey
 - i. Here is the survey link:
<https://redcap.musc.edu/surveys/?s=PKMDXE3AX3EFJEWI>
 - ii. Council can begin distribution immediately.
 - iii. Please email Kendall with where/to what groups the link has been distributed so we can track dissemination and not duplicate efforts**
 - iv. Karen Kemper to share information on and disseminate the needs assessment at the state-wide Patient Education Conference on April 13th
 - v. Other messaging and marketing materials to help further promote the needs assessment is forthcoming
 - vi. Ideas for dissemination?
 - b. SC Rare Disease website (scraredisease.org)
 - i. Working with MUSC web team for redesign. Open for content suggestions/ideas from council
 - ii. Idea to create Resources link for patients and families
 - c. Contact Registry
 - i. Discussion item to be followed up on post-symposium
 1. Follow up on Medicaid database utilization needed
 - d. SCTR to work with science writing intern to promote RDAC activities; Kendall and Tara will meet with her next week to discuss dissemination
 - e. RDAC will share a table with SCTR at the MUSC Senior Expo on March 13

5. Housekeeping and Additional Topics
 - a. Please send updated CVs and headshots to Kendall (spillman@musc.edu) by 02/01/24. Kendall to send reminder email
 - b. Swag representing SCTR and SCresearch.org will be ordered for symposium
 - i. Ordering items from preferred vendor Concorde LLC [Marianne Thomason, Mt. Pleasant | Promo - Home \(concordesc.com\)](#). Send any suggestions to Kendall (we will need to order in the next week or two)
 - c. Schedule next meeting
 - i. Kendall will be in touch to schedule a quarterly meeting for April
6. Resources
 - a. Reminder of Rare Disease Clinical Research Network website & newsletter [Subscribe to the Spotlight Newsletter | Rare Diseases Clinical Research Network \(rarediseasesnetwork.org\)](#)
 - b. Rare Disease Research Webinar to be held January 18. Registration link provided [How Gene Therapy Will Move Rare Disease Research at Warp Speed \(advarra.com\)](#)
7. Adjourn

Meeting Minutes: 04/12/24

Meeting Agenda:

1. Patrick Flume: Welcomed guest attendees; introduced NORD Policy Manager Carolyn Sheridan
2. Carolyn gave NORD SC State Report Card Presentation
 - a. Presentation provides evaluation of SC's effectiveness of serving the rare disease community within 9 target areas
 - i. Presentation link will be made available on SC RDAC website
 - c. Carolyn encourages members to attend April 25 Region C Policy & Advocacy Taskforce virtual meeting
 - d. Members can share the NORD SC State Report Card with elected officials by clicking on button in presentation pdf
 - e. SC legislation determines financial and health care policies that impact the rare disease community (and NORD's report card evaluation)
 - i. Dr. Flume would like to see where other states had success in these areas and discuss in a following meeting where to prioritize our efforts
 - ii. Carolyn will send Kendall copies of laws from nearby states
3. Rare Disease Day Symposium Debrief
 - a. Inaugural symposium was held February 29 at Segra Park to coincide with Rare Disease Day; 26 in-person attendees, 47 virtual attendees
 - b. Dr. Flume is pleased overall with the event turnout and balance of research and advocacy; open to suggestions for different locations and panel sessions for next year's event; would like to see more statewide involvement
 - c. Evaluation Feedback
 - i. Kendall provided feedback from 17 evaluation survey respondents collected so far; overall positive feedback
 1. Suggested Future Topics: patient panel; more information on clinical trials & statewide resources; more information on basic & translational research; speakers from diverse healthcare professions & roles they play in patient care; future treatment studies & curative therapies.
 - d. Council feedback
 - i. Guest attendee Sue Martin: attended symposium and found it valuable; would like higher attendance in future
 - ii. Divya Ahuja: a breakdown of the audience would be helpful in determining who attended as either a peer or relative or fellow advocate; consider hosting in an appealing region like Charleston next year for bigger draw
 - iii. Sue Martin: having a central location and keeping it affordable will help draw a larger audience; hosting it in Columbia can help draw elected officials
 - iv. Divya Ahuja: SC Telehealth Association hosts an annual conference in February or March he helps organize; SC RDAC could have a table there in future
4. Other council projects
 - a. Needs Assessment survey
 - i. Here is the survey link:
<https://redcap.musc.edu/surveys/?s=PKMDXE3AX3EFJEWI>

- ii. Kendall: currently 35 of the 61 recorded respondents have completed the survey; too few for thorough data analysis; a summary of findings will be available on the SC RDAC website once analyzed
 - iii. Assessment of why not all respondents completed the survey, or why such few numbers: literacy rates, rural areas, lack of resource accessibility for knowledge of survey
 - iv. Dr. Flume is seeking ideas for further dissemination
 - 1. Carolyn Sheridan has offered to share it on NORD
 - 2. Jonathan Hawayek: other states have used NORD's patient advocacy group list to disseminate surveys
 - 3. Sue Martin: has disseminated through her BDA newsletter and social media pages; suggests making a patient story or video about the survey's importance
 - 4. Taylor Dominguez: can share with RDDC and SC advocacy members; has offered to translate the survey into Spanish for Hispanic population
 - 5. Ginie Daguise: asks about its readability; Kendall says it was compiled for a lay public audience, but can review it
 - 6. Dr. Flume: survey was modified from other states' surveys; will reach out to contact in Colorado RDAC re: their survey responses for a benchmark
5. Housekeeping and Additional Topics
- a. Thoughts on replacing council member (Rare Disease Organization)
 - i. Dr. Flume requests that council members send suggestions
 - ii. Carolyn Sheridan offered to push notice of vacancy to patient advocacy groups
 - b. SC Rare Disease website (scraredisease.org)
 - i. Kendall is presenting a vision for website changes to Dr. Flume next week
 - ii. updates will take time due to gap in training level and cooperation with website management team; welcomes council feedback as updates are made
 - iii. Events page was added to site and Symposium event details and Agenda pdf were posted on it; landing page has today's meeting and link posted
 - c. Old Business
 - i. Dr. Flume: requested that Jon Hawayek submit list of top 50 rare diseases from Florida and Ginie Daguise submit from SC; plan is to obtain state data analytics
 - 1. Jon Hawayek: SPARK had commissioned outside consultant in Florida to provide top 50 of rare disease prevalence and provide ICD10 code and NDC code for treatment; now working on top 51-500 of rare disease prevalence
 - 2. Ginie Daguise: does not have access to data sets for SC; suggests contacting Dr. Anna Lopez -De Fede of Family Medicine Institute as she works with Medicaid data and could facilitate research request
 - ii. Jon Hawayek: most state employee health plans use PBM; Florida requested through PBM to pull data from state employee health plans
 - iii. Tripp Jennings: data would be available through RFA or revenue & fiscal affairs

- iv. Ginie Daguise: a number of programs provide funding, looking into possible costs
 - v. Kate Wink: thought state agencies could only charge for copies; not the case
 - vi. Dr. Flume: we could compile the data and then submit an analysis request and see if it's an allowable expense
 - d. Please send updated CVs and headshots to Kendall (spillman@musc.edu)
 - e. Kendall will send NORD State Report Card Presentation with meeting minutes in forthcoming email and add to website
 - f. Schedule next meeting
 - i. Kendall will be in touch to schedule a quarterly meeting for July
- 6. Adjourn

RARE DISEASE SYMPOSIUM



Attend in-person or virtually to learn about innovative and impactful research going on across the state, hear personal stories from those affected by rare disease and discover some of the important advocacy work that's grown out of both necessity and hope.

EVENT DETAILS

IN-PERSON LOCATION

SEGRA PARK

1640 FREED STREET
COLUMBIA, SC 29201

The symposium is free and open to the public but registration is strongly encouraged.

Boxed lunches will be provided to anyone attending in-person who registers on or before February 14th.



REGISTER NOW



**February 29,
2024**

10 am - 2 pm

rarediseaseSC.org

SC Rare Disease Symposium



February 29, 2024
Program Agenda

9:30 Check in / Registration

10:00 Welcome & Opening Remarks

Patrick Flume, MD, Endowed Chair, Power-Huggins Endowed Chair for Cystic Fibrosis & Chair for the Rare Disease Advisory Council

10:10 Clinical Trials & Patient Engagement

Patrick Flume, MD

10:30 Research Presentations: Innovation and Discovery within South Carolina

The Evolving Role of Genetic Testing for Rare Disorders

Dan Judge, MD, Director of the Cardiovascular Genetics Program & the Fellowship Director for Cardiovascular Disease, MUSC

The Changing Landscape of Newborn Screening: What's New and What's on the Horizon

Neena Champaigne, MD, Division Chief of Pediatric Genetics, MUSC

A Genetic Counselor's Role in Rare Disease

Maysen Mesaros, MS, CGC, Genetics Counselor for Neurology, MUSC & *Olivia Thompson, MS, CGC*, Genetics Counselor for Pediatrics, MUSC

Transforming Care and Research for Those with Rare Diseases: Start by Listening to Patients

Russell "Chip" Norris, PhD, Professor of Regenerative Medicine and Cell Biology, MUSC

12:00 Q&A Research Presentations

12:15 Break / Pick Up Lunch

12:30 Advocacy Panel

Moderator: Karen Kemper, PhD, Advocate for Scleroderma & Associate Professor of Department of Health Services, Clemson University

Yvonne Donald, Advocate for Sickle Cell Disease & Executive Director of the James R. Clark Memorial Sickle Cell Foundation

Miranda McManus, Advocate for Neurofibromatosis & Member of the Directors Council for the Response Evaluation in Neurofibromatosis and Schwannomatosis International Collaboration

Kerri Nelson, Advocate for Mosaic Trisomy 22 & Former Ambassador of the National Organization of Rare Diseases South Carolina Rare Action Network

LaKesha Williams, Advocate for Scleroderma & Support Group Facilitator for the South Carolina Piedmont Chapter of the National Scleroderma Foundation

1:30 Q&A Advocacy Panel

1:50 Wrap Up/Closing Remarks





2024 STATE REPORT CARD SOUTH CAROLINA

April 12, 2024

Carolyn G. Sheridan, MPH
State Policy Manager, East



NORD[®]
National Organization
for Rare Disorders

Alone we are rare. Together we are strong.[®]

National Organization for Rare Disorders Mission Statement

We improve the health and well-being of people with rare disease by driving advances in care, research and policy.



Driving Life-Saving Policy

NORD is the only independent and nonpartisan U.S. organization working at the intersection of care, research, policy, and community for all rare diseases.

We work with national and state decision-makers to advance the following priorities:

- New and Better Therapies
- Access and Affordability of Prescription Drugs
- Comprehensive Health Insurance
- Expanding Telehealth Services
- Strengthening Policies to Support Timely Diagnosis



as of November 2023

NORD's State Report Card[®]

- Since 2015, on an annual basis, NORD has evaluated how effectively states are serving people with rare diseases across 9 issue areas:

| | |
|--|------|
| Medicaid Financial Eligibility | C |
| Medical Nutrition | F |
| Newborn Screening | B |
| Prescription Drug Out-of-Pocket Costs | C |
| Protecting Patients in State Medicaid Programs | F |
| Protecting Patients in State Regulated Insurance | D |
| Rare Disease Advisory Council | YES! |
| Step Therapy (Fail First) | F |
| Telehealth | FAIL |

REPORT CARD “F” CATEGORIES

Medical Nutrition
Protecting Patients in Medicaid (1115)
Step Therapy
Telehealth



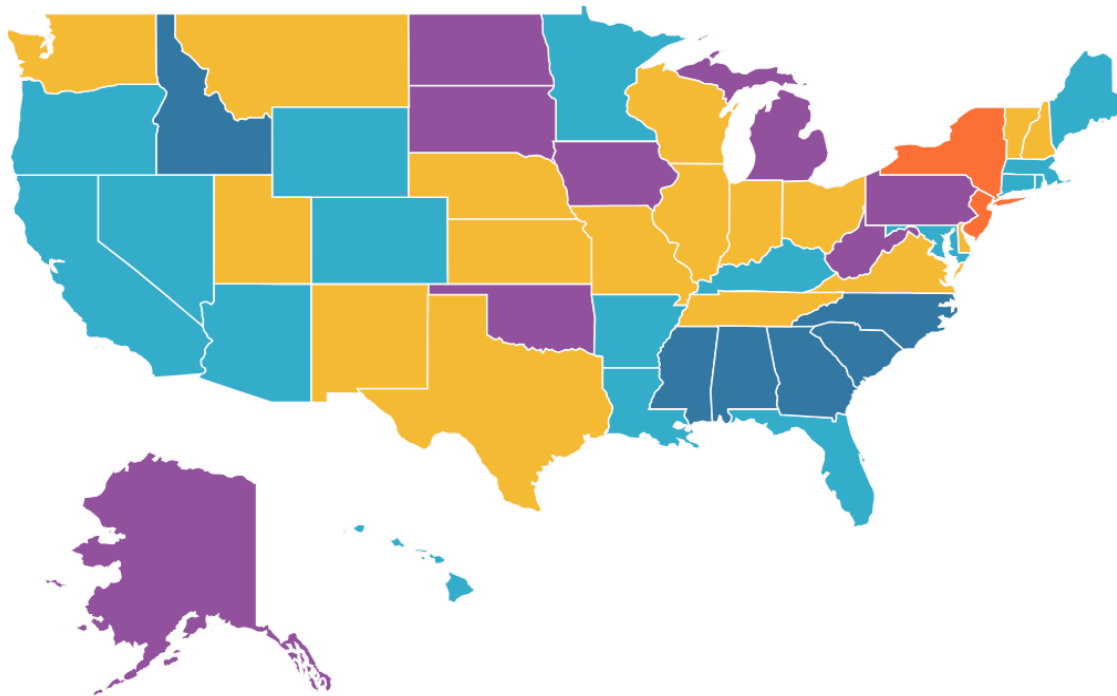
NORD[®]
National Organization
for Rare Disorders

Alone we are rare. Together we are strong.[®]

Medical Nutrition Grading Methodology

“a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation.”

Overall Grade ■ A ■ B ■ C ■ D ■ F



Grade state on four separate categories:

SC as of: November 2023

| | |
|---|----------|
| Coverage Requirements in Commercial Health Plans | F |
| Covered Disorders Requirements in Commercial Health Plans | F |
| Coverage Requirements in State Plans | F |
| Covered Disorders in State Plans | F |

and an **OVERALL** state grade is determined by taking the average of the four separate grades.

Medical Nutrition in South Carolina

State government can act to ensure insurance plans are providing coverage for medical nutrition. In South Carolina, this area of law is a **blank slate** and there is NO legislation pending in the current session year.

What Would Legislation Elevating South Carolina to an “A” Look Like?

- Mandate state regulated insurance companies and Medicaid provide coverage for the cost of enteral formulas for home use, whether administered orally or via tube feeding, for which a physician or other licensed health care provider authorized to prescribe has issued a written order.
 - NO limit on route of administration, age or monetary restrictions.
 - NO limit on conditions which may be eligible.

Protecting Patients in State Medicaid Programs

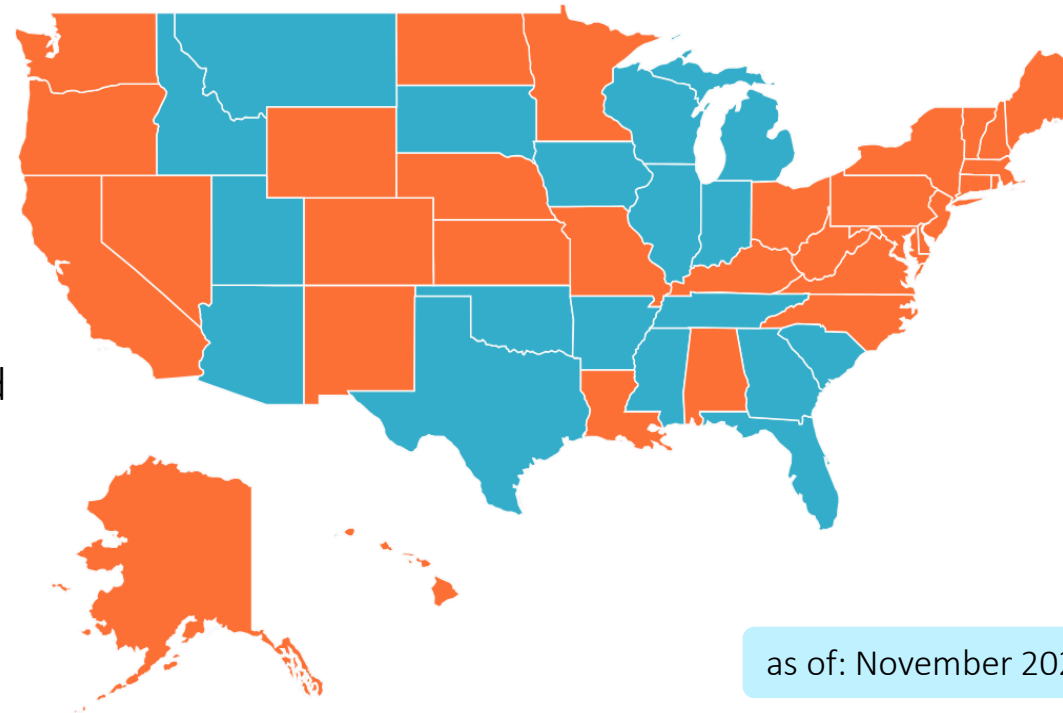
Medicaid Section 1115 Waivers

Section 1115 of the Social Security Act allows states to request authority to tailor their Medicaid programs. Many State Medicaid programs use these “1115 waivers” to allow the state to deviate from some federal Medicaid rules regarding eligibility and benefits.

Pass Fail

This area is graded on a “Pass” or “Fail” basis considering:

- Complexities and differences amongst state Medicaid programs;
- Status of each waiver; and
- Harm posed by proposed policies.



FAIL
If a state is working to implement or is currently seeking a waiver that contains policies that are less helpful to the rare disease community.

PASS
The state is not making efforts to implement or seek to receive a waiver for policies that are less helpful to the rare disease community.

as of: November 2023

Medicaid Section 1115 Waivers in South Carolina

On December 12, 2019, CMS approved two separate 1115 waivers for South Carolina which would extend Medicaid coverage from 67% to 100% FPL for its parent/caretaker relative groups and a new targeted adult group with initial and continued enrollment conditioned on compliance with work requirements at the regular match rate.

- This coverage did not qualify as a full Medicaid expansion under the ACA.

NOTABLE WAIVER INFORMATION

August 23, 2018 | SC Submits Application for Transition to Preconception Care (*still pending*)

August 10, 2021 | CMS Rescinds Approval of SC Healthy Connections Works

September 14, 2021 | CMS Rescinds Approval of SC Palmetto Pathways to Independence

Step Therapy Grading Methodology

Step therapy is a process by which insurers (public or private) require patients to try one or more alternative medications or treatments, and fail on those, before they can access the medicine or form of care initially ordered by their provider.

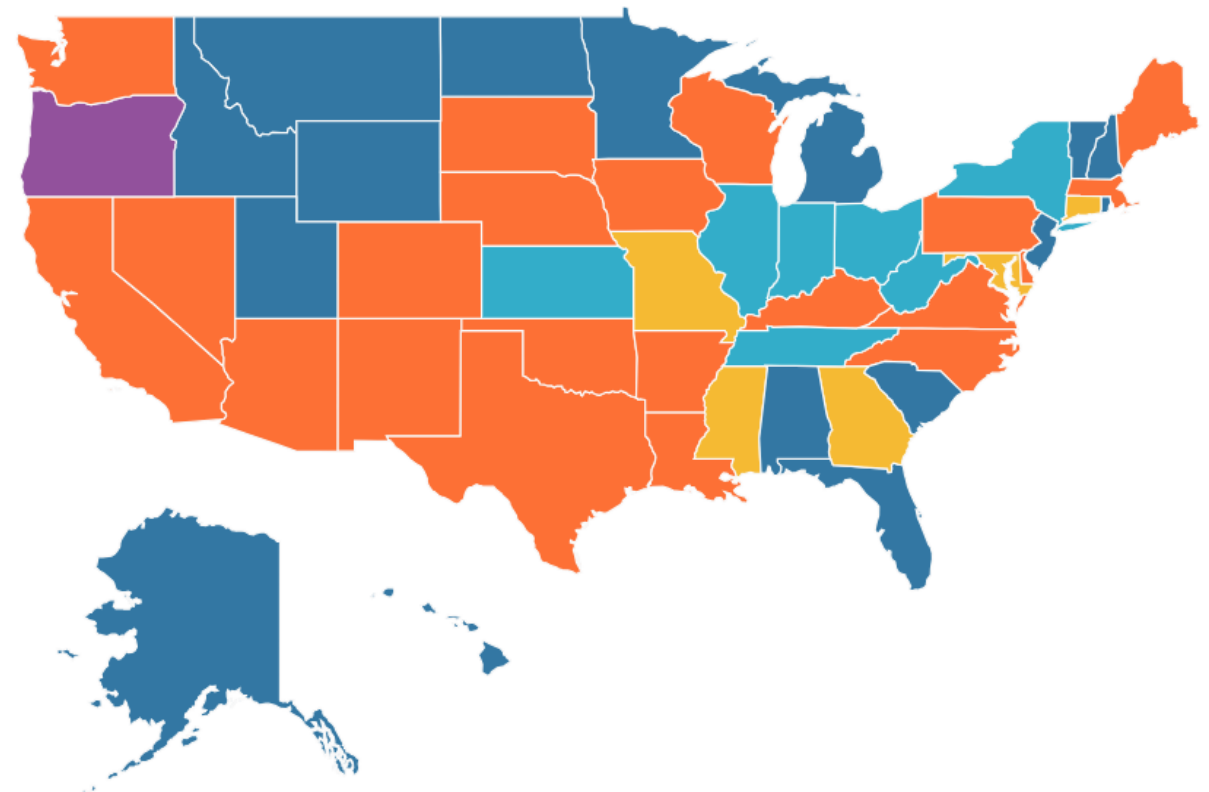
NORD considers how states have instituted protections around the use of step therapy:

SC as of: November 2023

| | |
|--------------------------------------|----------|
| Protocols Based on Clinical Practice | F |
| Timelines for Plan Response | F |
| Clarity of the Exceptions Process | F |
| Categories of Exceptions | F |

an **OVERALL** state grade is determined by taking the average of the four separate grades.

Overall Grade: ■ A ■ B ■ C ■ D ■ F



Step Therapy in South Carolina

Over half the states in the country have enacted protections for patients and regulation around the practice of step therapy. In South Carolina, this area of law is another **blank slate** and there is NO legislation pending in the current session year.

What Would Legislation Elevating South Carolina to an “A” Look Like?

| Regulate Protocols | Establish Categories for Exemptions from Protocols | Set Timelines for Responses to Requests from an Exemption |
|---|--|---|
| <p>Require protocols be based upon medical criteria and clinical guidelines developed by independent experts and consider the needs of atypical populations.</p> | <p>Allow the provider or patient to request an exemption if any one of the following apply:</p> <ol style="list-style-type: none"> 1) Rx is contraindicated 2) Rx is expected to be ineffective 3) The patient has already tried and failed – even if on different insurance 4) Rx is not in the best interest of the patient. 5) The patient is stable on the medicine the provider ordered initially. | <p>Establish a clear timelines for a response from the health plan</p> <p>URGENT → 72-Hours NON-URGENT → 24-Hours</p> |

Telehealth in South Carolina and Legislation

Both the federal government and state governments have the power to regulate aspects of telehealth including health provider licensure requirements, reimbursement rates and eligible services. There are several areas of South Carolina law that dictate the practice of telehealth or telemedicine – most notably *Title 40, Chapter 42 of the Code of South Carolina*.

“Telehealth or “telemedicine” is the use of any electronic communication, information technology or other means of delivering care between a provider in one location and a patient in another.

“Telemedicine” means the practice of medicine.

“Telehealth” includes clinical health care, patient and professional health-related education, public health, or health administration.

Telehealth Considerations for Discussion

In 2020, at the peak of the COVID-19 Pandemic, the telehealth landscape saw numerous changes nationwide and state-by-state. Recognizing the importance of telehealth to the rare disease community, NORD drafted [principles](#) to guide our engagement on the issue.

Access Should be Equal &
Effective for All

Patient Choice Must be
Prioritized



Ensure Transparency
Regarding Privacy Protection
& Cost Sharing

Decision Making Must Be
Data-Driven

REPORT CARD “C” AND “D” CATEGORIES

Medicaid Financial Eligibility
Rx Out of Pocket Costs
Protections in State Marketplace (STLDI)



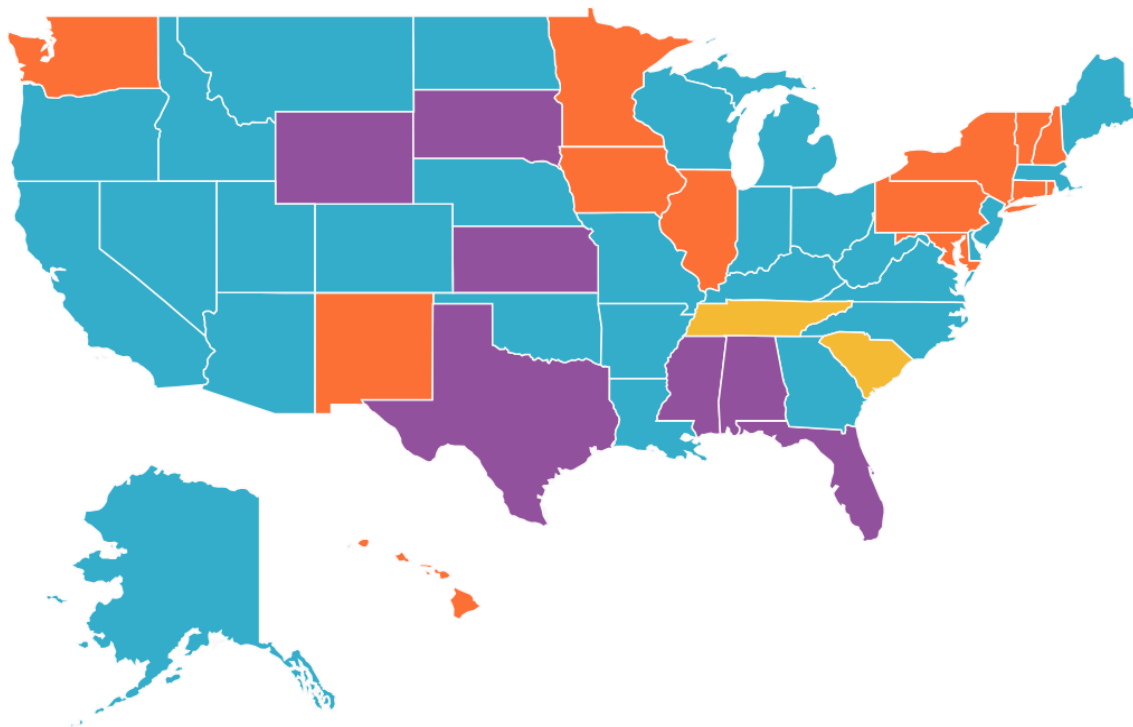
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Alone we are rare. Together we are strong.[®]

Medicaid Financial Eligibility

Medicaid is a joint federal and state program that helps cover medical costs for some people with limited income. South Carolina's Medicaid Program is called *Healthy Connections*.

Overall Grade: ■ A ■ B ■ C ■ D ■ F



Grades states on four separate categories:

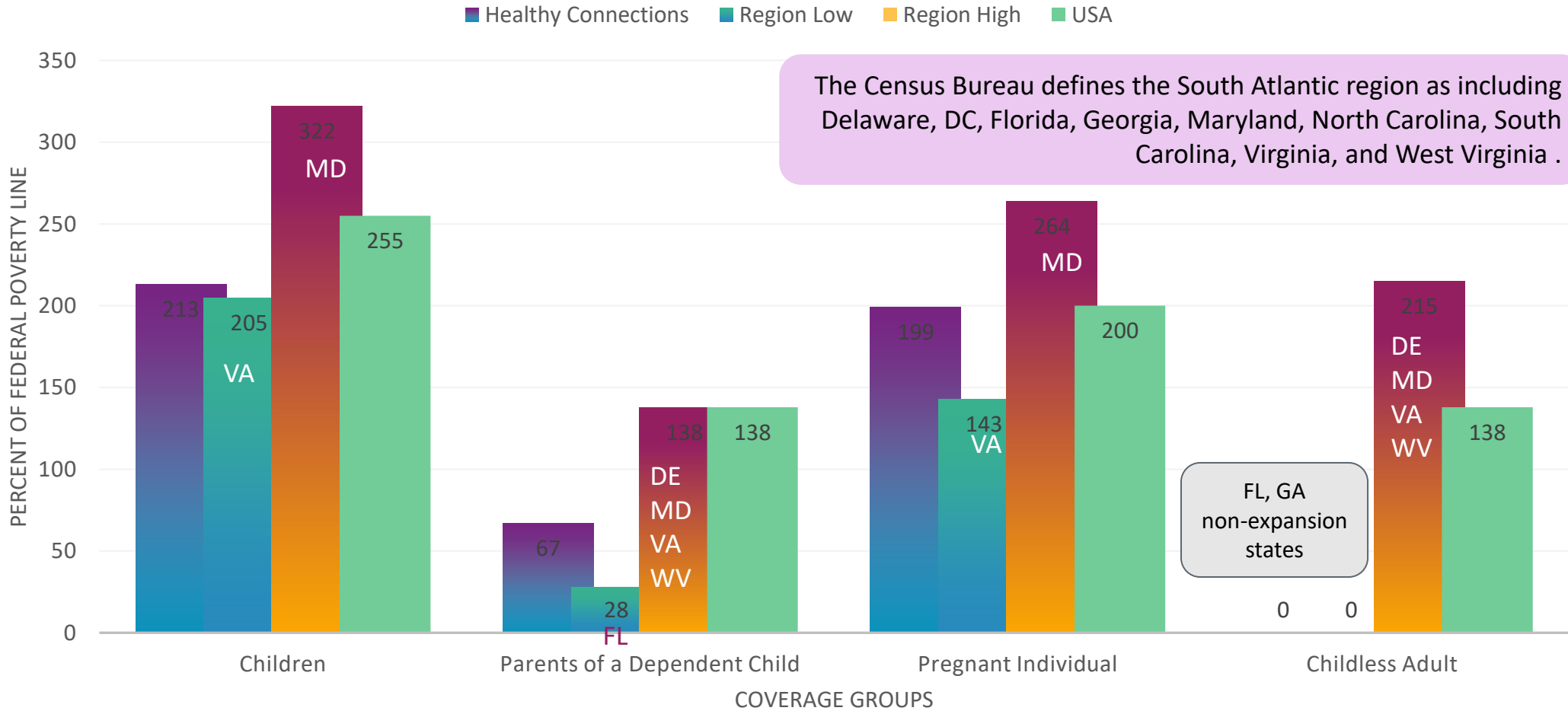
SC as of: November 2023

| | |
|---|----------|
| Eligibility for Parents of Dependent Children | D |
| Eligibility for Childless Adults | F |
| Eligibility for Pregnant Women | B |
| Eligibility for Children (incl. CHIP-Funded) | B |

and an **OVERALL** state grade is determined by taking the average of the four separate grades.

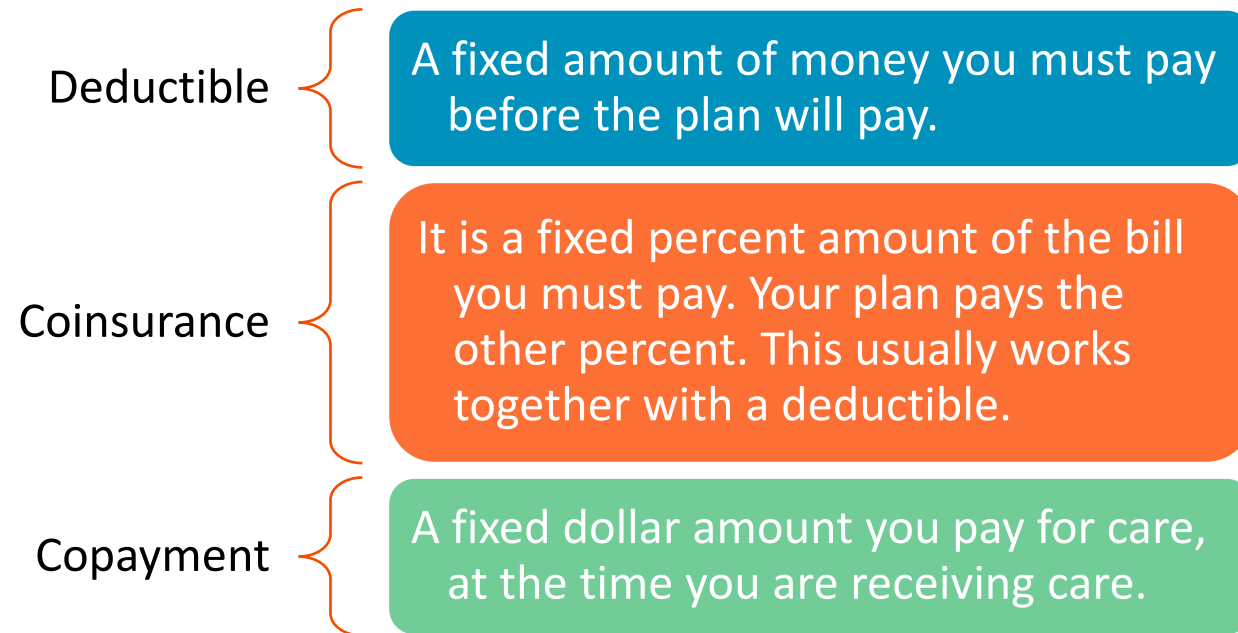
Medicaid Financial Eligibility in South Carolina

Healthy Connections Eligibility for Certain Groups Compared to Region Lows/Highs and USA



Prescription Drug Out-of-Pocket Costs

Out-of-Pocket costs are the expenses for medical care that are NOT paid for by your insurance company. Your plan may use one of any combination of these forms of “cost-sharing”



What Can SC Do?

- Deductible Protections** – Prohibit or limit deductibles for Rx drug costs.
- Drug Cost Caps** - Cap the amount a patient pays per month per drug or create a fixed copay.
- ✓ **Standardize Benefit Plan Design** – Make it easier to compare plans by making sure plans offer standard options.
- Separate Annual Drug Costs** – Place a limit on the total amount a patient pays on Rx drugs annually.
- Ban Copay Accumulators** – Ensure all payments to count towards a patient’s deductible – no matter the source.

Protecting Patients in State-Regulated Insurance

Short-Term, Limited-Duration Health Plans

S

- What are short-term, limited-duration health plans (STLDIs)?

These plans were designed for people who experience a temporary gap in health insurance coverage. These plans provide coverage for a limited term, typically less than 365 days.

T

- How else is that different from typical health insurance?

STLDI is exempt from the definition of individual health insurance coverage under the Public Health Service Act and, therefore, is generally not subject to the applicable federal individual market consumer protections and requirements for comprehensive coverage under the ACA. For example, STLDIs are exempt from rules that prohibit medical underwriting, pre-existing condition exclusions, and lifetime and annual limits, and that require minimum coverage standards.

L

- So, how are these plans regulated?

The Federal Department of Health & Human Services has a [rule](#) in place regarding duration and renewal of these plans. However, some states also have laws on the books related to these plans. **South Carolina Department of Insurance permits a plan that provides benefits for no longer than 11 months and may be renewed.**

D

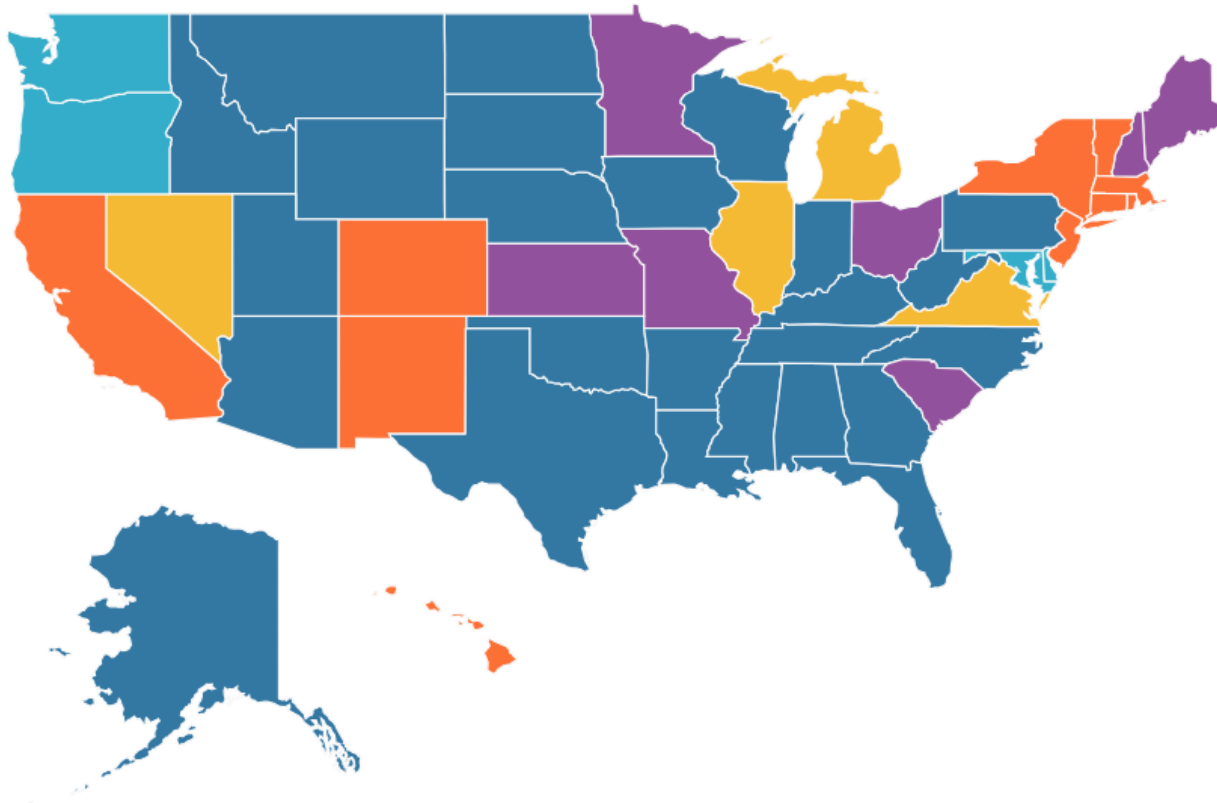
- What does the HHS rule require?

Current rules allow for a STLDI to initially be issued for 364 days, permit renewals of the same policy up to 3 times, and permit individuals to purchase additional STLDI policies.

I

STLDI Plans in South Carolina

Overall Grade: ■ A ■ B ■ C ■ D ■ F



NORD considers what actions, if any, states have taken to mitigate the expansion of short-term, limited-duration health plans.

SC as of: November 2023

| | |
|---------------------------------|----------|
| Limits on Initial Plan Duration | D |
| Regulation of Renewability | F |
| Limitations on Maximum Duration | D |

an **OVERALL** state grade is determined by taking the average of the three separate grades.

REPORT CARD “A” AND “B” CATEGORIES

Newborn Screening
Rare Disease Advisory Councils (RDAC)



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RDAC News & Opportunities to Engage

SAVE THE DATE!

April 25, 2024 | Region C Policy and Advocacy Taskforce Meeting (Virtual)

June 7 -8, 2024 | Living Rare, Living Stronger Patient and Family Forum in Los Angeles, CA

June 8, 2024 | Rare Impact Awards in Los Angeles, CA

October 20-22, 2024 | NORD Breakthrough Summit in Washington, DC

Mississippi RDAC Bill Approved by Governor Tate Reeves!

On Monday, April 8th NORD's eastern region welcomed a new member to the RDAC family! Governor Tate Reeves approved SB2156, championed by Senator Kevin Blackwell, going into law. There will be a bill signing event in Jackson next week!

Take Action!
Share South Carolina's Report Card



**Tell Your Elected Officials
About How South Carolina
Did on the State Report Card**



Share your state's grade on rare disease policies with your elected officials.

Take Action!

Thank You!

Carolyn Sheridan, MPH

State Policy Manager, East

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