HOUSE OF REPRESENTATIVES STAFF FINAL BILL ANALYSIS

BILL #: HB 7085 PCB HHS 24-04 Sickle Cell Disease
SPONSOR(S): Health & Human Services Committee, Skidmore and others
TIED BILLS: **IDEN./SIM. BILLS:** SB 7070

FINAL HOUSE FLOOR ACTION: 116 **Y's** 0 **N's** **GOVERNOR'S ACTION:** Approved

SUMMARY ANALYSIS

HB 7085 passed the House on February 28, 2024, and subsequently passed the Senate on March 6, 2024.

Sickle cell disease (SCD) is the most common inherited blood disorder in the United States, affecting approximately 100,000 Americans. SCD affects mostly, but not exclusively, Americans of African ancestry. SCD is a group of inherited disorders in which abnormal hemoglobin cause red blood cells to buckle into the iconic sickle shape; the deformed red blood cells damage blood vessels and over time contribute to a cascade of negative health effects beginning in infancy, such as intense vaso-occlusive pain episodes, strokes, organ failure, and recurrent infections. The severity of complications generally worsens as people age, but treatment and prevention strategies can mitigate complications and lengthen the lives of people with SCD.

Treatment for SCD has improved significantly in recent decades. Appropriate pharmaceutical treatments and evidence-based management protocols have the capacity to significantly improve the quality of life for people with SCD. In spite of the improvements in treatments for SCD, there significant underutilization among patients, due in part to gaps in understanding of the disease and its treatments among health care practitioners.

In 2023, the Legislature directed the Department of Health (DOH) to partner with a community-based sickle cell disease medical treatment and research center to establish and maintain a registry to track outcome measures of newborns who are identified as carrying a sickle cell hemoglobin variant. Adults identified as carrying a sickle cell hemoglobin variant are not eligible to participate in the registry.

HB 7085 creates the Sickle Cell Disease Research and Treatment Grant Program (Program) within DOH. Under the Program, the Office of Minority Health and Health Equity, within DOH, will award grants to community-based sickle cell disease medical treatment and research centers to fund the operation of Centers of Excellence for the treatment of sickle cell disease and the development of a health care workforce that is prepared to address the unique needs of patients with sickle cell disease.

The bill expands the existing sickle cell registry to allow adults with sickle cell disease to, at their discretion, opt into the registry.

The bill has a significant, negative fiscal impact on DOH. The bill has no fiscal impact on local government.

The bill was approved by the Governor on May 31, 2024, ch. 2024-225, L.O.F., and will become effective on July 1, 2024.

I. SUBSTANTIVE INFORMATION

A. EFFECT OF CHANGES:

Background

Sickle Cell Disease

Sickle cell disease (SCD) affects approximately 100,000 Americans, making it the most common inherited blood disorder in the United States.[1] SCD affects mostly, but not exclusively, Americans of African ancestry.[2] SCD encompasses a group of inherited disorders in which abnormal hemoglobin cause red blood cells to buckle into the iconic sickle shape; the deformed red blood cells damage blood vessels and over time contribute to a cascade of negative health effects beginning in infancy, such as intense vaso-occlusive pain episodes, strokes, organ failure, and recurrent infections.[3]

The severity of complications from SCD generally worsen as people age, but treatment and preventative strategies can mitigate complications, improve quality of life, and lengthen the lifespan of people with SCD.[4] SCD was historically perceived as a childhood disease due to high rates of childhood mortality, however, more than 90 percent of those living with the disease today are expected to survive into adulthood.[5] Roughly 60 percent of individuals with SCD in the United States today are adults, but the life expectancy of such individuals remains approximately 22 years shorter than the general population.[6]

Management of SCD

Management of SCD primarily focuses on treating and preventing complications caused by the disease such as acute pain episodes, infection, stroke, vision loss, and severe anemia. The most well-researched treatments for SCD relate to mitigating the risk of infection and stroke in children. There is a lack of research-driven data specific to adult populations with SCD.[7]

Stroke is one of the most common and devastating complications of SCD.[8] Blood transfusions may be used to treat acute episodes of elevated stroke risk, or through chronic transfusion therapy which reduces a person's overall stroke risk and prevents painful vaso-occlusive events.[9] Chronic transfusion

[1] National Heart, Lung, and Blood Institute, *What is Sickle Cell Disease?* Available at https://www.nhlbi.nih.gov/health/sickle-cell-disease (last visited January 30, 2024).
[2] Centers for Disease Control and Prevention, *Data & Statistics on Sickle Cell Disease*. Available at https://www.cdc.gov/ncbddd/sicklecell/data.html (last visited January 30, 2024).
[3] Centers for Disease Control and Prevention, *What is Sickle Cell Disease?* Available at https://www.cdc.gov/ncbddd/sicklecell/facts.html (last visited January 24, 2024). See also, AHCA (2023) *Florida Medicaid Study of Enrollees with Sickle Cell Disease*. Available at https://ahca.myflorida.com/content/download/20771/file/Florida_Medicaid_Study_of_Enrollees_with_Sickle_Cell_Disease.pdf (last visited January 24, 2024).
[4] Centers for Disease Control and Prevention, *Complications of Sickle Cell Disease*. Available at https://www.cdc.gov/ncbddd/sicklecell/complications.html (last visited January 24, 2024).
[5] DiMartino, L. D., Baumann, A. A., Hsu, L. L., Kanter, J., Gordeuk, V. R., Glassberg, J., Treadwell, M. J., Melvin, C. L., Telfair, J., Klesges, L. M., King, A., Wun, T., Shah, N., Gibson, R. W., Hankins, J. S., & Sickle Cell Disease Implementation Consortium (2018). *The sickle cell disease implementation consortium: Translating evidence-based guidelines into practice for sickle cell disease.* American journal of hematology, 93(12), E391–E395. https://doi.org/10.1002/ajh.25282.
[6] Lubeck D, Agodoa I, Bhakta N, et al. (2019) *Estimated Life Expectancy and Income of Patients With Sickle Cell Disease Compared With Those Without Sickle Cell Disease*. JAMA Netw Open. 2019;2(11):e1915374. doi:10.1001/jamanetworkopen.2019.15374. Available at https://jamanetwork.com/journals/jamanetworkopen/article-abstract/2755485 (last visited January 30, 2024).
[7] Adams-Graves, P. & Bronte-James, L. *Recent Treatment Guidelines for Managing Adult Patients with Sickle Cell Disease: Challenges in Access to Care, Social Issues, and Adherence*. (2016). Expert Review of Hematology, 9:6, 511-614. http://dx.doi.org/10.1080/17474086.2016.1180242
[8] U.S. Department of Health and Human Services, National Heart, Lung, and Blood Institute. *Evidence-Based Management of Sickle Cell Disease: Expert Panel Report* (2014). Available at https://www.nhlbi.nih.gov/health-topics/evidence-based-management-sickle-cell-disease (last visited January 31, 2024).
[9] Brandow, A.M., Panepinto, J.A. (2010). *Hydroxyurea Use in Sickle Cell Disease: The Battle with Low Prescription Rates, Poor Patient Compliance, and Fears of Toxicities*. Expert Reviews: Hematology. DOI: 10.1586/EHM.10.22

therapy has been shown to improve health-related quality of life in children with SCD.[10] There are, however, risks associated with frequent blood transfusions and chronic transfusion therapy can be logistically and financially difficult for caregivers to manage.[11] A transcranial Doppler ultrasound (TCD), is a specialized ultrasound device capable of detecting elevated stroke risk.[12] For children ages 2-16 with SCD who have a heightened risk of stroke, annual TCD screening is recommended by the American Society of Hematology to monitor stroke risk and prevent stroke.[13]

People with SCD are generally at a higher risk of severe bacterial infections due to poor spleen function, but fatality is especially high among young children and infants who lack the immune response necessary to combat infection. Defective or reduced spleen function begins early in the first year of life for infants with SCD.[14] To protect against life-threatening pneumococcal bacterial infection, daily oral penicillin is the standard of care for children from infancy through age five.[15]

In addition to daily oral penicillin and routine screening to monitor stroke risk in children, there are other pharmaceutical treatments available to manage the symptoms of SCD, reduce the long-term health impacts of the disease, and improve quality of life for children and adults with SCD. Hydroxyurea is an oral medication taken once daily which has been proven to be effective at reducing a person's pain episodes, mitigating stroke risk, and preventing organ damage.[16] Hydroxyurea is generally safe for both children and adults and is recommended for patients with certain forms of SCD experiencing "frequent pain episodes" or acute chest syndrome.[17]

Opioids are commonly used to treat the severe acute pain that results from vaso-occlusive episodes. Opioids are not recommended for treatment of the chronic pain that is associated with SCD due to the significant risks of overdose and addiction associated with frequent opioid use. Opioids are, however, highly effective at managing acute severe pain in acute settings and as such the National Heart Lung and Blood Institute recommends rapid initiation of opioids for patients visiting the emergency department for a vaso-occlusive pain episode.[18]

More recent pharmaceutical developments for the treatment of SCD include L-glutamine, Voxelotor, and Crizanlizumab. L-glutamine in an essential amino acid which was approved by the FDA in 2017 for the treatment of SCD in adults and children over five years of age. The mechanism of action of L-glutamine is not well understood, however, it has been shown to reduce a patient's number of sickle cell crisis episodes.[19] Voxelotor and Crizanlizumab are two disease modifying drugs approved by the

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[10] Beverung, L.M., Strouse, J.J., Hulbert, M.L. (2015) *Health-related Quality of Life in Children with Sickle Cell Anemia: Impact of Blood Transfusion Therapy*. American Journal of Hematology. http://doi.org/10/1002/ajh.2387

[11] *Supra*, note 12.

[12] Runge, A., Brazel, D., Pakbaz, Z. (2022). *Stroke in Sickle Cell Disease and the Promise of Recent Disease Modifying Agents*. Journal of the Neurological Sciences. http://doi.org/10.1016/j.jns.2022.120412

[13] DeBaun, M., et al. *American Society of Hematology 2020 guidelines for sickle cell disease: prevention, diagnosis, and treatment of cerebrovascular disease in children and adults*. (2020). *Blood Advances*; 4 (8): 1554–1588. doi: https://doi.org/10.1182/bloodadvances.2019001142

[14] U.S. Department of Health and Human Services, National Heart, Lung, and Blood Institute. *Evidence-Based Management of Sickle Cell Disease: Expert Panel Report* (2014). Available at https://www.nhlbi.nih.gov/health-topics/evidence-based-management-sickle-cell-disease (last visited January 31, 2024).

[15] AHCA (2023) *Florida Medicaid Study of Enrollees with Sickle Cell Disease*. Available at https://ahca.myflorida.com/content/download/20771/file/Florida_Medicaid_Study_of_Enrollees_with_Sickle_Cell_Disease.pdf (last visited January 24, 2024). Amoxicillin may also be prescribed for this purpose. In patients with a known or suspected penicillin allergy, erythromycin is prescribed.

[16] *Id.*

[17] U.S. Department of Health and Human Services, National Heart, Lung, and Blood Institute. *Evidence-Based Management of Sickle Cell Disease: Expert Panel Report* (2014). Available at https://www.nhlbi.nih.gov/health-topics/evidence-based-management-sickle-cell-disease (last visited January 31, 2024).

[18] *Id. See also*, Smeltzer, M.P., Howell, K.E., Treadwell, M. (2021). *Identifying barriers to evidence-based care for sickle cell disease: results from the Sickle Cell Disease Implementation Consortium cross-sectional survey of healthcare providers in the USA*. BMJ Open 2021. DOI: 10.1136/bmjopen-2021-050880

[19] Quinn C. T. (2018). *l-Glutamine for sickle cell anemia: more questions than answers*. Blood, 132(7), 689–693. https://doi.org/10.1182/blood-2018-03-834440. *See also*, Ballas S. K. *The Evolving Pharmacotherapeutic Landscape for the Treatment of Sickle Cell Disease* (2020). Mediterranean Journal of Hematology and Infectious Diseases, 12(1), e2020010. https://doi.org/10.4084/MJHID.2020.010

FDA in 2019. The drugs may be beneficial for different subgroups of SCD patients for whom other treatments have proven insufficient or ineffective. Voxelotor and Crizanlizumab act through different mechanisms, but both mitigate the harmful effects that damaged red blood cells have on the body. There is ongoing research into their impact on other SCD morbidities.[20]

Curative Treatments for SCD

On December 8, 2023, the FDA approved the first two gene therapies for the treatment of SCD. The products, Casgevy and Lyfgenia, are cell-based gene therapies approved for the treatment of SCD in patients 12 years of age or older. Both products are made from the patients' own blood stem cells, which are modified, and administered to the patient as a one-time, single-dose infusion as part of a hematopoietic (blood) stem cell transplant. Prior to treatment, a patients' stem cells are collected, and then the patient must undergo high-dose chemotherapy, a process that removes cells from the bone marrow so they can be replaced with the modified cells.[21]

The recently FDA-approved gene therapies have not reached full market availability, but the costs are anticipated to be as high as $2 to million per patient.[22] It is yet to be determined how insurance companies or Medicaid will cover the treatment.[23]

Prior to the approval of these gene therapy treatments, the only treatment for SCD with curative potential was a matched/related hematopoietic stem cell transplant (HSCT). HSCT has been shown to be highly effective as a cure, though outcomes are more favorable when the transplant is performed before age 16 and with a matched sibling donor.[24] While highly curative, HSCT poses significant risks including transplant rejection that can result in the patient's death.[25] The procedure is infrequently performed due to the high cost,[26] the limited number of capable transplant centers, the strenuous preparation regimen and significant risks,[27] and the need for a genetically matched donor.[28]

Barriers to Care for SCD

While SCD is the most common inherited blood disorder in the U.S. and is often diagnosed at birth through newborn screening programs,[29] patients with SCD often experience significant barriers to

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[20] *Supra*, note 12.
[21] U.S. Food & Drug Administration, *FDA Approves First Gene Therapies to Treat Patients with Sickle Cell Disease* (2023). Available at https://www.fda.gov/news-events/press-announcements/fda-approves-first-gene-therapies-treat-patients-sickle-cell-disease (last visited January 30, 2024).
[22] National Heart, Lung, and Blood Institute. *FDA approval of gene therapies for sickle cell disease: Q&A with NHLBI Director Dr. Gary Gibbons and NHLBI's Division of Blood Diseases and Resources Director Dr. Julie Panepinto* (2023). Available at https://www.nhlbi.nih.gov/news/2023/fda-approval-gene-therapies-sickle-cell-disease-dr-gibbons-dr-panepinto (last visited January 30, 2024).
[23] MacMillan, C., *Casgevy and Lyfgenia: Two Gene Therapies Approved for Sickle Cell Disease* (2023). Yale Medicine. Available at https://www.yalemedicine.org/news/gene-therapies-sickle-cell-disease (last visited January 30, 2024).
[24] Gluckman, E., Cappelli, B., Bernaudin, F., et al. (2017). *Sickle cell disease: an international survey of results of HLA-identical sibling hematopoietic stem cell transplantation*. Blood, 129(11), 1548–1556. https://doi.org/10.1182/blood-2016-10-745711
[25] Ashorobi D, Bhatt R. *Bone Marrow Transplantation in Sickle Cell Disease*. (2022). In: StatPearls. Treasure Island (FL): StatPearls Publishing. Available at https://www.ncbi.nlm.nih.gov/books/NBK538515/ (last visited January 31, 2024).
[26] *Supra*, note 17. HSCT is estimated to cost approximately $1 million to $2 million per person.
[27] *Supra*, note 17.
[28] Salcedo, J., Bulovic, J., & Young, C. *Cost-effectiveness of a Hypothetical Cell or Gene Therapy Cure for Sickle Cell Disease* (2021). Scientific Reports. https://doi.org/10.1038/s41598-021-90405-1
[29] Centers for Disease Control and Prevention. *Newborn Screening (NBS) Data* (2023). Available at https://www.cdc.gov/ncbddd/hemoglobinopathies/scdc-state-data/newborn-screening/index.html#:~:text=Newborn%20screening%20(NBS)%20for%20sickle,SCD%20living%20in%20a%20state. (last visited January 20, 2024).

accessing adequate care. Barriers include lack of health insurance, unmet transportation needs, a shortage of relevant specialists, and a lack of familiarity with SCD among health care professionals. There is a limited number of knowledgeable health care professionals with expertise in the management of SCD, and mistrust among patients and bias among providers continue to affect access to and quality of care.[30]

Recent decades have brought major scientific advancements in understanding the biological mechanisms of SCD, the development of new pharmaceutical treatments, the establishment of evidence-based treatment protocols, and methods for mitigating the risk of catastrophic complications.[31] Collectively, these advancements provide the means for significantly improving the health and quality of life for many patients with SCD; however, few of these interventions are utilized to their full potential.

The nature of SCD inherently leads to a greater use of health care services compared to the general population, but gaps in access to appropriate care are common and lead to unmitigated health crises and an increased utilization of costly emergency medical services.[32] Health care practitioners who have not specialized in the treatment of SCD express discomfort in prescribing essential treatments for SCD,[33] and demonstrate a lack of knowledge regarding recent treatment developments.[34]

Access to adequate care is especially challenging for young adults who are transitioning from pediatric to adult care settings.[35] While SCD has historically been associated with childhood mortality, more than 90 percent of those living with the disease are expected to survive into adulthood today.[36] The system of care for SCD has developed with a focus on pediatric patients; as a result, patients with SCD are more likely to receive well-managed preventative care as children through specialized pediatric programs. Patients aging out of pediatric care and transitioning into adult care are less likely to have access to consistent and appropriate care for the treatment of SCD, which leads to an increased dependence on emergency medical care compared to other age groups.[37]

Emergency care settings present additional challenges for patients treating patients with SCD. Vaso-occlusive pain crises are the most common reason a person with SCD will seek emergency medical treatment, but such crises are poorly understood by many emergency care professionals and highly stigmatized. Patients who present for emergency care in the midst of a vaso-occlusive pain crises may have their behavior perceived as drug seeking and have the severity of their pain doubted and undertreated.[38] Educational gaps and biases among providers, staff, and patients create barriers to

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[30] Sickle Cell Disease Coalition, *State of Sickle Cell Disease: 2020 Report Card* (2020). Available at http://www.scdcoalition.org/pdfs/SCD%20Report%20Card%202020.pdf (last visited January 31, 2024).
[31] American Society of Hematology. *ASH Sickle Cell Disease Initiative: Sickle Cell Disease Timeline*. Available at https://www.hematology.org/advocacy/sickle-cell-disease-initiative/scd-timeline (last visited January 30, 2024).
[32] DiMartino, L. D., Baumann, A. A., Hsu, L. L., Kanter, J., Gordeuk, V. R., Glassberg, J., Treadwell, M. J., Melvin, C. L., Telfair, J., Klesges, L. M., King, A., Wun, T., Shah, N., Gibson, R. W., Hankins, J. S., & Sickle Cell Disease Implementation Consortium (2018). *The sickle cell disease implementation consortium: Translating evidence-based guidelines into practice for sickle cell disease.* American Journal of Hematology, 93(12), E391–E395. https://doi.org/10.1002/ajh.25282. *See also*, Brousseau, D.C., Owens, P.L., Mosso, A.L., Panepinto, J.A., Steiner, C.A. *Acute Care Utilization and Rehospitalizations for Sickle Cell Disease* (2010). JAMA. 2010;303(13):1288–1294. doi:10.1001/jama.2010.378
[33] Lanzkron S, Haywood C Jr, Hassell KL, Rand C. *Provider barriers to hydroxyurea use in adults with sickle cell disease: a survey of the sickle cell disease adult provider network*. (2008) Journal of the National Medical Association. 100(8): 968-973. https://doi.org/10.1016/S0027-9684(15)31419-X
[34] Robinson, K., Esqro, R., Cooper, S., LoPresti, M., & Carson, B. *Identifying and Addressing Knowledge and Confidence Gaps Regarding the Management of Patients with Sickle Cell Disease Via Engaging Continuing Medical Education*. (2023). *Blood,* 142 (Supplement 1): 7228. doi: https://doi.org/10.1182/blood-2023-177576
[35] Hemker, B., Brouseau, D., Yan, K., Hoffmann, R., & Panepinto. *When Children with Sickle Cell Disease Become Adults: Lack of Outpatient Care Leads to Increased Use of the Emergency Department* (2011). American Journal of Hematology. 86:10, 863-865. https://doi.org/10.1002/ajh.22106
[36] *Id*.
[37] Blinder, M. A., Duh, M. S., Sasane, M., Trahey, A., Paley, C., & Vekeman, F. *Age-Related Emergency Department Reliance in Patients with Sickle Cell Disease* (2015). The Journal of Emergency Medicine, 49(4), 513–522.e1. https://doi.org/10.1016/j.jemermed.2014.12.080
[38] DiMartino, L. D., Baumann, A. A., Hsu, L. L., Kanter, J., Gordeuk, V. R., Glassberg, J., Treadwell, M. J., Melvin, C. L., Telfair, J., Klesges, L. M., King, A., Wun, T., Shah, N., Gibson, R. W., & Hankins, J. S. *The sickle cell disease implementation consortium:*

communication and trust, and erode the provider–patient relationship, which can result in inadequate or inappropriate treatment of patients.[39]

Florida's Medicaid SCD Population

In 2022, the Legislature directed the Agency for Health Care Administration (AHCA) to conduct a study assessing Florida's population of Medicaid enrollees with SCD and their utilization of specific health care services.[40] The Florida Medicaid Study of Enrollees with Sickle Cell Disease (the study) analyzed data from 2018 through 2021 and found that Florida's rate of Medicaid enrollees with SCD was twice that of the national average,[41] with approximately 7,328 Medicaid enrollees with SCD per year. The study found that Florida's Medicaid SCD population was predominantly female (58%), young (median age 18), and Black (63%).

The study showed that nearly all of the Medicaid SCD population received treatment from a physician at least once during the study period. 85 percent of Medicaid SCD patients were evaluated or treated in an outpatient clinic setting, 61 percent were treated in an emergency room (ER) at least once, and 52 percent were admitted for inpatient care in a hospital. Individuals who received treatment in an ER had an average of 4.5 visits to the ER during the four-year study period.

The study showed that routine screenings and preventative treatments were broadly underutilized by the Medicaid SCD population. Only 41 percent of children in the Medicaid SCD population had at least one TCD screening for stroke risk during the four-year study period; this is significantly less than the recommended annual screening for children with SCD.[42] Data on blood transfusions, which are commonly used to reduce stroke risk when elevated risk is detected by TCD, were not included in the study.

The study showed that penicillin was the most commonly prescribed SCD-relevant medication for Medicaid SCD patients. The study showed that 58 percent of eligible individuals were being prescribed penicillin, but there remains a persistent gap between use and recommended care. Other medications for treating SCD symptoms and complications were prescribed with even less frequency. Hydroxyurea[43] and L-glutamine were prescribed to only 22 percent and 2 percent of eligible SCD Medicaid patients respectively. The newer disease-modifying drugs, Voxelotor and Crizanlizumab were each prescribed to less than 1 percent of the eligible Medicaid SCD population.

Sickle Cell Disease Registry

SCD presents unique challenges to medical researchers; patient data is scare, small sample sizes limit research possibilities, and mistrust of the medical establishment is common among this patient population. Patient registries are a means to overcome some of the research limitations that exist due to the nature of SCD. Patient registries are organized systems that allow for the use of observational study methods to collect uniform data and evaluate specified outcomes for a population defined by a particular disease.[44]

Translating evidence-based guidelines into practice for sickle cell disease (2018). American Journal of Hematology, 93(12), E391–E395. https://doi.org/10.1002/ajh.25282

[39] Glassberg, G., *Improving Emergency Department-Based Care of Sickle Cell Pain* (2017). Hematology. American Society of Hematology. Education Program, 2017(1), 412–417. https://doi.org/10.1182/asheducation-2017.1.412

[40] AHCA (2023) *Florida Medicaid Study of Enrollees with Sickle Cell Disease*. Available at https://ahca.myflorida.com/content/download/20771/file/Florida_Medicaid_Study_of_Enrollees_with_Sickle_Cell_Disease.pdf (last visited March 13, 2024).

[41] Centers for Medicare and Medicaid Services (2021), *Medicaid and CHIP Sickle Cell Disease Report, T-MSIS Analytic Files (TAF) 2017*. Available at https://www.medicaid.gov/medicaid/quality-of-care/downloads/scd-rpt-jan-2021.pdf (last visited March 13, 2024).

[42] *Supra*, note 17.

[43] AHCA cites high-cost as a potential barrier to the utilization of hydroxyurea by patients, however, hydroxyurea is on Florida's preferred drug list for patients with SCD, which significantly reduces the cost for Medicaid patients.

[44] Hageman, I.C., van Rooij, I.A., de Blaauw, I., et al. *A systematic overview of rare disease patient registries: challenges in design, quality management, and maintenance* (2023). Orphanet Journal of Rare Diseases. https://doi.org/10.1186/s13023-023-02719-0

In 2023, the Legislature directed the Department of Health (DOH) to partner with a community-based sickle cell disease medical treatment and research center to establish and maintain a registry to track outcome measures of newborns who are identified as carrying a sickle cell hemoglobin variant.[45] DOH has since contracted with the Foundation for Sickle Cell Research for the implementation of the registry.[46] Under current law, only newborns who have been detected as carrying a sickle cell hemoglobin variant through the Newborn Screening Program are included in the registry. Parents may choose to have their child removed from the registry by submitting a form provided by DOH.[47] There is not a mechanism under current law for adults with SCD to be included in the registry. Current law also directs the newborn's primary care physician to provide the parent or guardian of the newborn with information regarding the availability and benefits of genetic counseling.

Effect of the Bill

Sickle Cell Disease Research and Treatment Grant Program

HB 7085 creates the Sickle Cell Disease Research and Treatment Grant Program (Program) within DOH. The Program will fund projects that improve the quality and accessibility of appropriate health care for Floridians with SCD. The Program seeks to:

- Improve the health outcomes and quality of life for Floridians with SCD;
- Expand access to high-quality, specialized care for SCD; and
- Improve awareness and understanding among health care practitioners of current best practices for the treatment and management of SCD.

The Office of Minority Health and Health Equity, within DOH, will award grants to community-based sickle cell disease medical treatment and research centers for projects which support the cultivation of a health care workforce that is educated and familiar with the unique needs of patients with SCD, and the growth and development of SCD Centers of Excellence. To be eligible for funds, a SCD Center of Excellence must be a health care facility dedicated to the treatment of patients with SCD which provides evidence-based, comprehensive, patient-centered coordinated care.

The bill requires DOH to:

- Publicize the availability of funds and establish an application process for grant proposals;
- Initiate a call for applications no later than July 15, 2024;
- Develop uniform data reporting requirements in order to evaluate the performance of grant recipients and the improvement of health outcomes; and
- Develop a monitoring process to evaluate progress toward meeting grant objectives.

DOH must also submit an annual report to the Governor, the President of the Senate, the Speaker of the House of Representatives, and the State Surgeon General by March 1 of each year. The report must include the status and progress of each project supported by the Program in the previous calendar year, and include the following components for each project:

- A summary of the project and the project outcomes or expected project outcomes
- The status of the project, including whether it has concluded or the estimated date of completion;
- The amount of the grant awarded and the estimated or actual cost of the project;

[45] S. 383.147, F.S.
[46] Department of Health. *Contract Summary: Contract # CMO28*. On file with the Healthcare Regulation Subcommittee.
[47] S. 383.147, F.S.

- The source and amount of any federal, state, or local government grants or donations or private grants or donations funding the project; and
- A list of all entities involved in the project.

The bill specifies that no more that 5 percent of grant funds may be used by a grant recipient toward administrative expenses. The bill also grants that the balance of any appropriation from the General Revenue Fund for the program which has not been disbursed, but which is obligated under a contract or committed to be expended June 30th of the fiscal year, may be carried forward for up to five years after the effective date of the original appropriation.

The bill authorizes DOH to adopt rules as necessary to implement the Program.

Sickle Cell Disease Registry

The bill creates a process by which adults with SCD who are Florida residents to choose to be included in the registry. The bill directs DOH to prescribe by rule the process for an adult to opt into the registry.

The bill also revises the registry to clarify the role of screening providers, DOH, and primary care physicians in the processes in current law. The bill transfers the responsibility of informing parents of the availability and benefits of genetic counseling from the infant's primary care physician to DOH.

The bill provides an effective date of July 1, 2024.

II. FISCAL ANALYSIS & ECONOMIC IMPACT STATEMENT

A. FISCAL IMPACT ON STATE GOVERNMENT:

1. Revenues:

   None.

2. Expenditures:

   The Sickle Cell Disease Research and Treatment Grant Program will have a significant, negative fiscal impact on DOH, dependent upon the amount of funds allocated to the program. The Fiscal Year 2024-2025 General Appropriations Act appropriated $10 million from the General Revenue Fund to DOH for sickle cell treatment and research grants and aid.[48]

B. FISCAL IMPACT ON LOCAL GOVERNMENTS:

1. Revenues:

   None.

2. Expenditures:

   None.

C. DIRECT ECONOMIC IMPACT ON PRIVATE SECTOR:

[48] Fiscal Year 2024-2025, General Appropriations Act, Conference Report for House Bill 5001, line 430a.

None.

D. FISCAL COMMENTS:

None.