HOUSE OF REPRESENTATIVES STAFF ANALYSIS

BILL #: HB 1043 Medicaid Coverage of Rapid Whole Genome Sequencing
SPONSOR(S): Anderson
TIED BILLS: **IDEN./SIM. BILLS:** SB 616

REFERENCE	ACTION	ANALYST	STAFF DIRECTOR or BUDGET/POLICY CHIEF
1) Healthcare Regulation Subcommittee		Poche	McElroy
2) Health Care Appropriations Subcommittee			
3) Health & Human Services Committee			

SUMMARY ANALYSIS

Genetic disorders are a leading contributor to morbidity and mortality in the neonatal and pediatric intensive care units in the United States. Approximately 7 percent to 10 percent of the 4 million infants born in the U.S. each year are admitted to a neonatal ICU for the diagnosis and treatment of an acute illness. About one percent of all neonatal ICU admissions have an indication of a genetic disorder, and approximately 15 percent of babies admitted to high acuity units appear to have a genetic disorder.

All organisms have a unique genetic code, or genome, comprised of nucleotide bases. If the sequence of the bases in an organism are known, its unique DNA fingerprint is known. Determining the order of bases is called sequencing. Whole genome sequencing is a laboratory procedure that determines the order of bases in the genome of an organism in one process. Rapid whole genome sequencing (rWGS) completes such sequencing quickly, and can produce a much quicker diagnosis.

HB 1043 requires the state Medicaid program to cover rWGS as a fee-for-service benefit for Medicaid recipients who:

- Are 21 years of age or younger;
- Have a complex or acute illness of unknown etiology that has not been caused by environmental exposure, toxic ingestion, an infection with normal response to treatment, or trauma; and
- Are receiving inpatient treatment in a hospital ICU or high-acuity pediatric care unit.

The bill restricts the use of any genetic data resulting from rWGS only to assist in diagnosing and treating the patient, and considers such data protected health information under the Health Insurance Portability and Accountability Act. The bill permits genetic data generated by rWGS to be used in scientific research only if the patient, or the patient's guardian if he or she is a minor, expressly consents to such use. Such consent may be rescinded at any time.

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

The bill provides an effective date of July 1, 2023.

FULL ANALYSIS

I. SUBSTANTIVE ANALYSIS

A. EFFECT OF PROPOSED CHANGES:

Background

Genetic Disorders

Genetic disorders are a leading contributor to morbidity and mortality in the neonatal and pediatric intensive care units in the United States. There are more than 13,000 known genetic diseases and hundreds of targeted treatments and orphan drugs that have been approved or are in clinical trials.[1] Approximately 7 percent to 10 percent of the 4 million infants born in the U.S. each year are admitted to a neonatal ICU for the diagnosis and treatment of an acute illness.[2] About 1 percent of all neonatal ICU admissions have an indication of a genetic disorder, and approximately 15 percent of babies admitted to high acuity units appear to have a genetic disorder, leading to longer hospitalizations and higher resource use.[3] Disease can progress rapidly in acutely ill infants, necessitating timely diagnosis in the hope of implementing personalized interventions that can decrease morbidity and mortality.[4]

Rapid Whole Genome Sequencing (rWGS)

All organisms have a unique genetic code, or genome, comprised of nucleotide bases (A, T, C, and G). If the sequence of the bases in an organism are known, its unique DNA fingerprint is known. Determining the order of bases is called sequencing. Whole genome sequencing is a laboratory procedure that determines the order of bases in the genome of an organism in one process.[5]

Scientists conduct whole genome sequencing by following these four main steps:

- DNA shearing: Scientists begin by using molecular scissors to cut the DNA, which is composed of millions of bases (A's, C's, T's and G's), into pieces that are small enough for the sequencing machine to read.
- DNA bar coding: Scientists add small pieces of DNA tags, or bar codes, to identify which piece of sheared DNA belongs to which bacteria. This is similar to how a bar code identifies a product at a grocery store.
- DNA sequencing: The bar-coded DNA from multiple bacteria is combined and put in a DNA sequencer. The sequencer identifies the A's, C's, T's, and G's, or bases, that make up each bacterial sequence. The sequencer uses the bar code to keep track of which bases belong to which bacteria.
- Data analysis: Scientists use computer analysis tools to compare sequences from multiple bacteria and identify differences. The number of differences can tell the scientists how closely related the bacteria are, and how likely it is that they are part of the same outbreak.

[1] S Kingsmore, L Smith, *A genome sequencing system for universal newborn screening, diagnosis, and precision medicine for sever genetic diseases*, Amer. J Human Genetics, vol. 109, pgs. 1605-1619, Sept. 1, 2022; M Clark, A Hildreth, Diagnosis of genetic diseases in seriously ill children by rapid whole-genome sequencing and automated phenotyping and interpretation, Sci. Transl. Med., vol. 11, 2019.
[2] D Dimmock, S. Caylor, et al., *Project Baby Bear: Rapid precision care incorporating rWGS in 5 California children's hospitals demonstrates improved clinical outcomes and reduced costs of care*, Amer. J. Human Genetics, vol. 108, pgs. 1-8, July 1, 2021, available at https://pubmed.ncbi.nlm.nih.gov/34089648/.
[3] Id.
[4] B Peterson, EJ Hernandez, *Automated prioritization of sick newborns for whole genome sequencing using clinical natural language processing and machine learning*, Genome Medicine, vol. 15:18, 2023.
[5] Centers for Disease Control and Prevention, *Whole Genome Sequencing*, available at https://www.cdc.gov/pulsenet/pathogens/wgs.html#:~:text=Whole%20genome%20sequencing%20is%20a%20fast%20and%20affordable,needed%20to%20quickly%20solve%20and%20prevent%20foodborne%20outbreaks (last viewed on March 30, 2023).

rWGS can complete such sequencing quickly and produce a much quicker diagnosis in as many as 50% of children.[6] With a turnaround as fast as 48 hours, vital time can be gained to develop and implement a plan of care for individuals, mostly children, with genetic disorders.

As the use of whole genome sequencing expands, CDC's national surveillance systems and laboratory infrastructure must keep pace with the changing technology. With modernization, CDC and its public health partners can continue to successfully detect, respond to, and stop infectious diseases.

Project Baby Manatee

From August 2019 to June 2020, Nicklaus Children's Hospital implemented a pilot program called Project Baby Manatee. Through the program, 50 patients were enrolled and sequenced using rWGS.[7] The average age was 34.6 months and two thirds were male, while 60 percent of participants were white, and 26 percent were African American.[8] Two types of testing was completed – rWGS and ultra rWGS. Ultra WGS testing was selected when delivery of the genetic diagnoses was critical for clinical management. Twenty out of fifty patients, or 40 percent, received genetic diagnoses based of WGS.[9] The most common presentations of illness in which a genetic disease was diagnosed by rWGS were respiratory problems (36 percent), cardiovascular problems (36 percent), seizures (32 percent), brain disorders (32 percent), and metabolic issues (24 percent).[10]

Eight of the diagnosed genetic diseases have an incidence of less than one in one million births or is of unknown incidence. Some of the identified diseases are so rare that many treating physicians had never seen them before, increasing the probability that these disorders would generally go underdiagnosed without rWGS. The rWGS led to changes in clinical management of 38 percent of children in the pilot program. Such results empowered clinicians and parents to quickly make informed decisions that typically altered the course of the child's hospitalization and led to the initiation of new procedures and medications, or the avoidance of unnecessary ones.[11] The program resulted in estimated savings of over $3.76 million, yielding a $2.88 million return on investment.[12] Based on the results of Project Baby Manatee, rWGS and related rapid precision medicine approach was cost-saving and cost-effective, but also improved health outcomes and shortened the period to diagnosis.[13]

Other Studies

Studies performed in clinical research settings have found genome sequencing to be effective for diagnosis and management of undiagnosed infants in ICUs and to result in improved diagnostic yield[14], faster time to diagnosis, enhanced physician and parental satisfaction, improved patient outcomes, and reductions in health care costs.[15] Further, rWGS facilitates end-of-life care decisions that can alleviate suffering and aid the grieving process.[16] Studies have shown rWGS provides a diagnosis for 21 percent to 57 percent of children in intensive care settings.[17]

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[6] University of California San Francisco, News and Events, *Rapid Whole Genome Sequencing: Faster Diagnostics for Fragile Infants*, May 14, 2020, available at https://precisionmedicine.ucsf.edu/news/rapid-whole-genome-sequencing-faster-diagnostics-fragile-infants (last viewed on March 30, 2023).
[7] Nicklaus Children's Hospital, *Project Baby Manatee – Advanced Genomics for Critically Ill Children Final Report*, Aug. 1, 2019 to June 30, 2020, available at https://www.nicklauschildrens.org/NCH/media/docs/pdf/research/Final-report-State-Appropriations-NCH-PMI.PDF.
[8] Id., at pg. 6.
[9] Id., at pg. 7.
[10] Id., at pg. 8.
[11] Id.
[12] Id., at pg. 19. Total costs for WGS and related rapid precision medicine for 50 patients was approximately $880,000.
[13] Id., at pg. 20.
[14] V Diaby, A Babcock, et al., *Real-world economic evaluation of prospective rapid whole-genome sequencing compared to a matched retrospective cohort of critically ill pediatric patients in the United States*, Pharmacogenomics J., vol. 22, pgs. 223-229, 2022.
[15] C Bupp, E Ames, et al., *Breaking Barriers to Rapid Whole Genome Sequencing in Pediatrics: Michigan's Project Baby Deer*, Children, vol. 10, pg. 106, 2023, available at https://doi.org/10.3390/children10010106; TA Lavelle, X Feng, et al., *Cost-effectiveness of exome and genome sequencing for children with rare and undiagnosed conditions*, Genet. Med., vol. 24, pgs.1349-1361, 2022.
[16] L Farnaes, A Hildreth, et al., *Rapid whole-genome sequencing decreases infant morbidity and cost of hospitalization*, npj Genomic Medicine 3:10, 2018.
[17] Id.

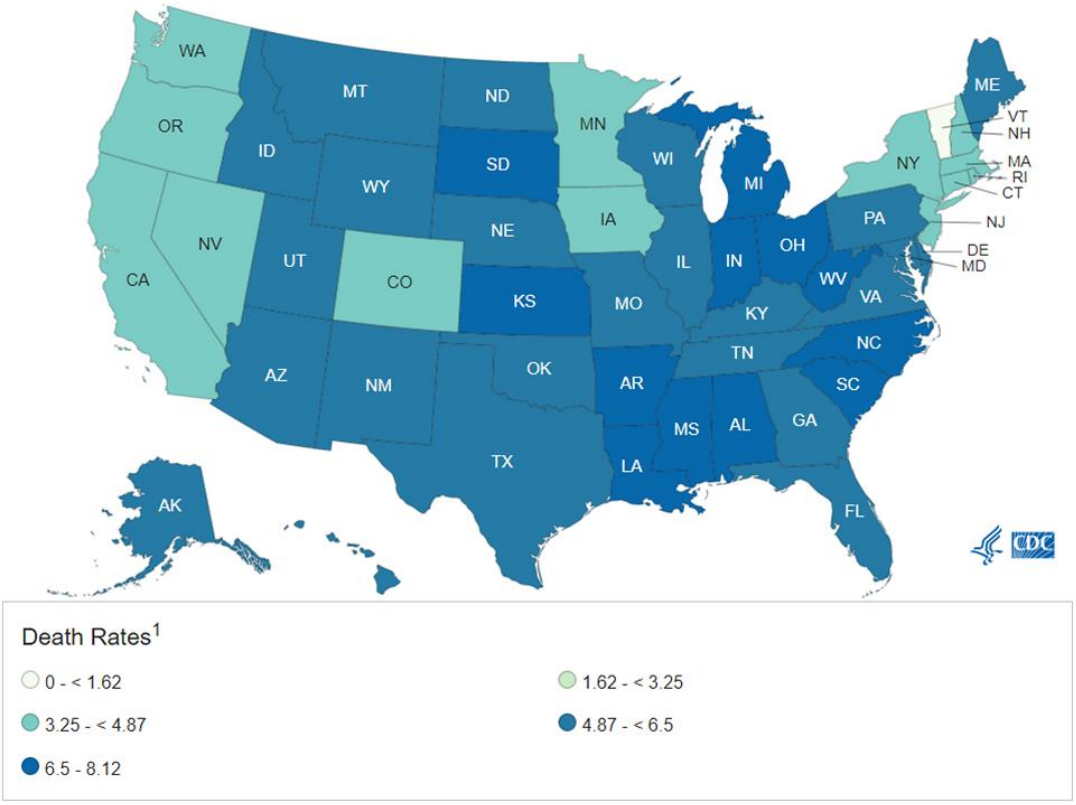
Despite evidence that sequencing improves clinical outcomes, reduce net costs of care, and leads to high provider and parental satisfaction, routine implementation in ICUs and coverage by payors has remained elusive.[18] It has been shown that WGS, while expensive, may decrease overall cost of diagnostic evaluations of medically complex children.[19]

Compared to traditional genetic tests, where diagnosis can be delayed or missed, rWGS has a high diagnostic yield, ranging from 40 percent to 70 percent.

Infant Mortality

Infant mortality is the death of an infant before his or her first birthday. The infant mortality rate is the number of infant deaths for every 1,000 live births. In 2020, the infant mortality rate in the United States was 5.4 deaths per 1,000 live births.[20]

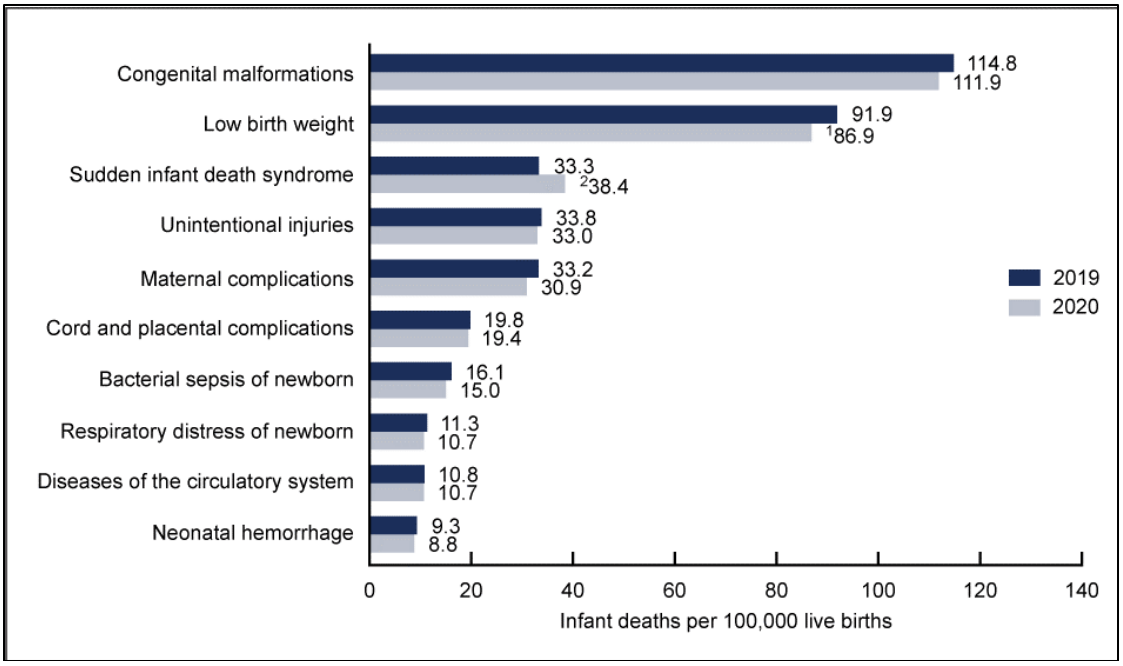
The following map shows infant mortality by state for 2020, the most recent year with available data. Florida's infant mortality rate is 4.96 to less than 6.62 per 1,000 live births.[21]



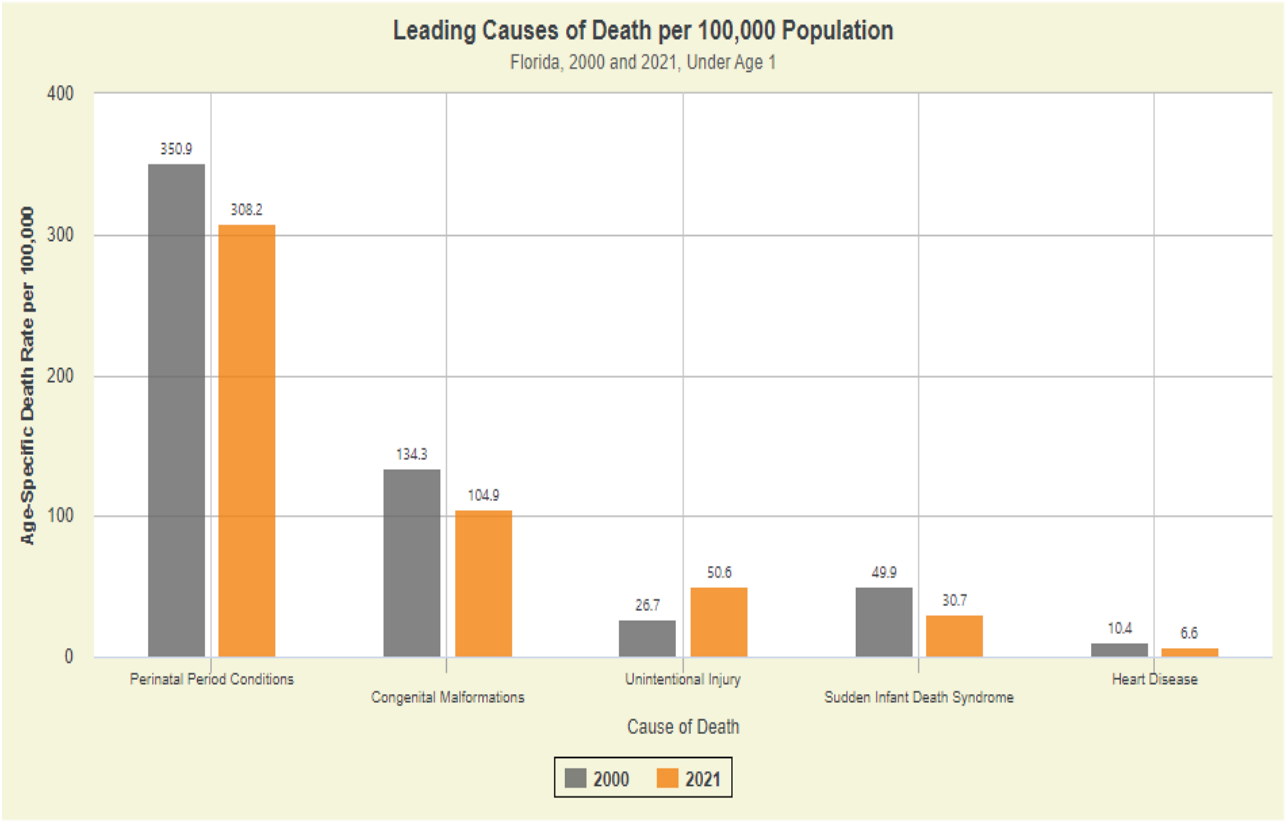
The 10 leading causes of infant death in 2020 (congenital malformations, low birth weight, sudden infant death syndrome, unintentional injuries, maternal complications, cord and placental complications, bacterial sepsis of newborn, respiratory distress of newborn, diseases of the circulatory system, and neonatal hemorrhage) accounted for 67.5% of all infant deaths in the United States. The graph below

[18] Supra, FN at pg. 2.
[19] Supra, FN 5 at pg. 2; R Hayeems, et al., *Care and cost consequences of pediatric whole genome sequencing compared to chromosome microarray*, Eur. J. Hum. Genet., vol. 25, pgs. 1303-1312, 2017.
[20] U.S. Department of Health and Human Services, Centers for Disease Control and Prevention, National Center for Health Statistics, Publication and Information Products–Data Briefs, *Mortality in the United States, 2020*, available at https://www.cdc.gov/nchs/products/databriefs/db427.htm#section_5 (last viewed on March 29, 2023).
[21] U.S. Department of Health and Human Services, Centers for Disease Control and Prevention, Reproductive Health, Maternal and Infant Health, *Infant Mortality Rates by State, 2020*, available at https://www.cdc.gov/reproductivehealth/maternalinfanthealth/infantmortality.htm (last viewed on March 29, 2023).

shows the IMR for the 10 leading causes of infant death in 2020 in the United States, including 2019 for comparison.[22]



The chart below shows the leading cause of death of infants in Florida for 2021, with 2020 rates included for comparison.[23]



[22] Supra, FN 1, figure 5.
[23] Florida Department of Health, Bureau of Community Health Assessment, Division of Public Health Statistics and Performance Management, Mortality Dashboard–Deaths by Age, *Leading Causes of Death per 100,000 Population, Florida, 2020 and 2021, Under Age 1*, available at https://www.flhealthcharts.gov/ChartsReports/rdPage.aspx?rdReport=MortalityAtlas.Dashboard_MortalityAtlas1&rdRequestForwarding=Form (last viewed on March 29, 2023).

Florida Medicaid

Medicaid is the health care safety net for low-income Floridians. Medicaid is a partnership of the federal and state governments established to provide coverage for health services for eligible persons. The program is administered by the Agency for Health Care Administration (AHCA) and financed by federal and state funds.

The structure of each state's Medicaid program varies, but what states must pay for is largely determined by the federal government, as a condition of receiving federal funds.[24] The federal government sets the minimum mandatory populations to be included in every program, and the minimum mandatory benefits to be covered. These mandatory benefits include physician services, hospital services, home health services, and family planning.[25] States can add benefits, with federal approval. Florida has added many optional benefits.[26]

The Florida Medicaid program covers approximately 5.7 million low-income individuals in Florida.[27] Medicaid is the largest single program in the state, representing more than 44 percent of the total Fiscal Year 2022-2023 state budget.[28] Florida's program is the 4th largest in the nation by enrollment and, for FY 2020-2021, the program is the 4th largest in terms of expenditures.[29]

Florida delivers medical assistance to most Medicaid recipients - approximately 78% - using a comprehensive managed care model.[30] A minority of Medicaid recipients-mostly those enrolled in limited benefit programs participate in the traditional fee-for-service model.

Payment for Genomic Testing

Florida's Medicaid program pays for the following standard, non-rapid genomic sequencing procedures:

- Fetal chromosomal aneuploidy genomic sequence analysis panel.
- Fetal chromosomal microdeletion genomic sequence analysis.
- Genomic sequencing procedures and other molecular multianalyte assays.
- Genetic testing for severe inherited conditions.[31]

Several states are currently implementing coverage policies for rWGS, including California[32], Minnesota[33], Louisiana[34], Maryland[35], and Oregon[36].

[24] Title 42 U.S.C. §§ 1396-1396w-5; Title 42 C.F.R. Part 430-456 (§§ 430.0-456.725).
[25] S. 409.905, F.S.
[26] S. 409.906, F.S.
[27] Agency for Health Care Administration, *Florida Statewide Medicaid Monthly Enrollment Report*, Feb. 2023, available at https://ahca.myflorida.com/medicaid/Finance/data_analytics/enrollment_report/index.shtml (last viewed on March 28, 2023). United States Census Bureau, *QuickFacts, Florida*, https://www.census.gov/quickfacts/fact/table/FL/PST045221 (last viewed on March 28, 2023).
[28] Ch. 2022-156, L.O.F. See also *Fiscal Analysis in Brief: 2022 Legislative Session*, available at http://edr.state.fl.us/content/revenues/reports/fiscal-analysis-in-brief/FiscalAnalysisinBrief2022.pdf (last viewed on March 28, 2023).
[29] The Henry J. Kaiser Family Foundation, *State Health Facts, Total Medicaid Spending FY 2021 and Total Monthly Medicaid and CHIP Enrollment Nov. 2022*, available at http://kff.org/statedata/ (last viewed on March 28, 2023).
[30] S. 409.964, F.S.
[31] Agency for Health Care Administration, *2023 Agency Legislative Administration–HB 1043*, pg. 2, March 7, 2023.
[32] Medi-Cal Update, *Inpatient Services, Bulletin 573*, June 2022, available at https://files.medi-cal.ca-gov/pubsdoco/bulletins/artfull/ips202206.aspx.
[33] Minnesota Department of Human Services, *Laboratory and Pathology Tests*, April 4, 2022, available at https://www.dhs.state.mn.us/main/idcplg?IdcService=GET_DYNAMIC_CONVERSION&RevisionSelectionMethod=LatestReleased&dDocName=DHS16_144353.
[34] Louisiana SB 154, *Provides for Health Insurance Coverage of Genetic Testing for Critically Ill Infants with No Diagnosis*, January 1, 2023, available at https://legisscan.com/LA/text/SB154/2022.
[35] Maryland Department of Health, Whole Genome Sequencing (WGS) Clinical Criteria, *Whole Genome Clinical Criteria*, available at https://health.maryland.gov/mmcp/Documents/Whole%20Genome%20Clinical%20Criteria.pdf#search=Whole%20Genome%20Sequencing%20Clinical%20Criteria.
[36] Oregon Health Authority, *Prioritized list of Health Services*, January 1, 2023, available at https://www.oregon.gov/oha/HPA/DSI-HERC/PrioritizedList/1-1-2022PrioritizedListofHealthServices.pdf.

Over the past decade, rWGS has developed into an effective diagnostic test for almost all heritable diseases and is gaining acceptance as a first-tier test for critically ill newborns with suspected genetic diseases.[37] There has been an increase in payor coverage for rWGS, but it varies and is not universal.

Effect of Proposed Changes

HB 1043 requires AHCA to cover rWGS as a fee-for-service benefit for Medicaid recipients who:

- Are 21 years of age or younger;
- Have a complex or acute illness of unknown etiology that has not been caused by environmental exposure, toxic ingestion, an infection with normal response to treatment, or trauma; and
- Are receiving inpatient treatment in a hospital ICU or high-acuity pediatric care unit.

The bill, by providing Medicaid coverage to children, will identify those children with serious genetic disorders in a timely manner, allowing those children to get treatment earlier in the disease process, likely reducing long-term medical costs for treating those children over the course of their lives.

The bill also restricts the use of any genetic data resulting from rWGS only to assist in diagnosing and treating the patient, and considers such data protected health information protected by the Health Insurance Portability and Accountability Act (HIPAA). It is likely this information is protected by HIPAA without this bill provision.

The bill permits genetic data generated by rWGS to be used in scientific research only if the patient, or the patient's guardian if he or she is a minor, expressly consents to such use. Such consent may be rescinded at any time. Upon receipt of written notice of consent rescission, the health care provider or entity using the genetic data must cease using it, and expunge the individual's genetic data from any data repository where it is held.

Lastly, the bill authorizes AHCA to seek approval to amend waivers, request a new waiver, and amend contracts as necessary to provide coverage of rWGS. AHCA is also given express rulemaking authority to implement the bill provisions.

The bill provides an effective date of July 1, 2023.

B. SECTION DIRECTORY:

Section 1: Creates s. 409.9063, F.S., relating to rapid whole genome sequencing services for Medicaid recipients.
Section 2: Provides an effective date of July 1, 2023.

II. FISCAL ANALYSIS & ECONOMIC IMPACT STATEMENT

A. FISCAL IMPACT ON STATE GOVERNMENT:

1. Revenues:

None.

2. Expenditures:

The bill has a significant, negative fiscal impact on the Medicaid program. In 2021, 14,476 newborn babies covered by Medicaid were admitted to a NICU facility. If 5 percent of those newborns were tested and testing was reimbursed as a fee-for-service benefit, 724 newborns would be tested. At a

[37] Supra, FN 1.

cost of $4,549.31 per test, the total potential expense is $3,292,791, with potential impact to General Revenue totaling $1,334,239. The chart below details potential expenses as the percentage of newborns tested increases. This projection is based on the number of newborn children in a NICU in 2021, and a reimbursement rate of 60% of the Medicare reimbursement rate for rWGS ($7,582.20 x 60%=$4,549.31).[38]

Participation	Potential Participants	Total Expense	GR Impact
5%	724	$3,292,791	$1,334,239
10%	1,448	$6,585,581	$2,668,477
15%	2,171	$9,878,372	$4,002,716
25%	3,619	$16,463,953	$6,671,194
50%	7,238	$32,927,906	$13,342,387
75%	10,857	$49,391,859	$20,013,581
100%	14,476	$65,855,812	$26,684,775

AHCA will need to determine reimbursement rates for Medicaid coverage of rWGS and include rWGS in the independent laboratory services fee schedule under Rule 59G-4.002, F.A.C. These actions can likely be implemented using existing resources.

B. FISCAL IMPACT ON LOCAL GOVERNMENTS:

1. Revenues:

None.

2. Expenditures:

None.

C. DIRECT ECONOMIC IMPACT ON PRIVATE SECTOR:

Entities that perform rWGS testing may see an increase in the number of tests.

D. FISCAL COMMENTS:

rWGS testing may lead to significant cost avoidance over the lifetime of an individual through early diagnosis and intervention before any disease conditions worsen. Such cost avoidance may offset the initial cost of rWGS testing.

III. COMMENTS

A. CONSTITUTIONAL ISSUES:

1. Applicability of Municipality/County Mandates Provision:

Not applicable. The bill does not impact municipal or county government.

2. Other:

[38] Agency for Health Care Administration, *2023 Agency Legislative Bill Analysis–HB 1043*, March 7, 2023, pg. 5.

None.

B. RULE-MAKING AUTHORITY:

The bill expressly provides AHCA with rulemaking authority to implement the bill provisions.

C. DRAFTING ISSUES OR OTHER COMMENTS:

The bill requires coverage for rapid whole genome sequencing for Medicaid recipients aged 21 years or younger. Current Medicaid policy defines children as individuals aged 20 years or younger, covering benefits up to the individual's 21st birthday. If the intent is to cover rapid whole genome sequencing for children in Medicaid, this provision of the bill should be changed to reflect current Medicaid eligibility and coverage parameters.

IV. AMENDMENTS/COMMITTEE SUBSTITUTE CHANGES