HOUSE OF REPRESENTATIVES STAFF ANALYSIS

BILL #: CS/CS/HB 1441 Department of Health
SPONSOR(S): Health & Human Services Committee, Health Care Appropriations Subcommittee, Anderson
TIED BILLS: **IDEN./SIM. BILLS:** SB 1582

REFERENCE	ACTION	ANALYST	STAFF DIRECTOR or BUDGET/POLICY CHIEF
1) Healthcare Regulation Subcommittee	19 Y, 0 N	Osborne	McElroy
2) Health Care Appropriations Subcommittee	14 Y, 0 N, As CS	Aderibigbe	Clark
3) Health & Human Services Committee	19 Y, 0 N, As CS	Osborne	Calamas

SUMMARY ANALYSIS

In the United States, a rare disease is any condition that nationally affects fewer than 200,000 people. There may be as many as 7,000 rare diseases impacting the lives of 25-30 million Americans and their families. So, while the individual diseases may be rare, the total number of people impacted by a rare disease is large.

Rare diseases include genetic disorders, infectious diseases, cancers, and various other pediatric and adult conditions. A rare disease can affect anyone at any point in their life, and can be acute or chronic. It is estimated that 80 percent or more of rare diseases are genetic. For genetic rare diseases, genetic testing is often the only way to make a definitive diagnosis. Rare diseases present a fundamentally different array of challenges compared to those of more common diseases; often patients are set on a “diagnostic odyssey,” in order to determine the cause of their symptoms as they seek treatment in health care settings where their condition may have never been seen before.

In 2023, the Legislature allocated $500,000 in General Revenue funds in the General Appropriations Act for pediatric rare disease research grants.

CS/CS/HB 1441 establishes the Andrew John Anderson Pediatric Rare Disease Grant Program (Program) within DOH with the purpose of advancing the progress of research and cures for rare pediatric diseases. The Program establishes grants for scientific and clinical research to further the search for new diagnostics, treatments, and cures for pediatric rare diseases.

Any university or established research institute located in this state may apply for a grant under the Program. The bill establishes a preference for grant proposals which foster collaboration among institutions, researchers, and community practitioners.

The bill requires DOH to award Program grants through a competitive, peer-reviewed process. The bill requires DOH to appoint peer review panels of independent, scientifically qualified individuals to review the scientific merit of each proposal and establish its priority score. DOH must provide the priority score to the RDAC which must consider the priority score in its recommendations for funding. DOH, after consultation with the RDAC, will award grants to selected applicants.

The bill has a significant, negative fiscal impact on state government. See *Fiscal Comments*. The bill has no fiscal impact on local governments.

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

FULL ANALYSIS

I. SUBSTANTIVE ANALYSIS

A. EFFECT OF PROPOSED CHANGES:

Background

Rare Diseases

In the United States, a rare disease is any condition that nationally affects fewer than 200,000 people. There may be as many as 7,000 rare diseases impacting the lives of 25-30 million Americans and their families.[1] So, while the individual diseases may be rare, the total number of people impacted by a rare disease is large.

Rare diseases include genetic disorders, infectious diseases, cancers, and various other pediatric and adult conditions. A rare disease can affect anyone at any point in their life, and can be acute or chronic. It is estimated that 80 percent or more of rare diseases are genetic. For genetic rare diseases, genetic testing is often the only way to make a definitive diagnosis. Rare diseases present a fundamentally different array of challenges compared to those of more common diseases; often patients are set on a “diagnostic odyssey,” in order to determine the cause of their symptoms as they seek treatment in health care settings where their condition may have never been seen before.[2]

In 2023, the Legislature allocated $500,000 in General Revenue funds in the General Appropriations Act for pediatric rare disease research grants.[3]

Rare Disease Advisory Council

The Legislature established the Rare Disease Advisory Council (RDAC) in 2021 to assist the Department of Health (DOH) in providing recommendations to improve health outcomes for individuals with rare diseases residing in the state.[4]

The establishment of RDACs across the country is an initiative spearheaded by the National Organization for Rare Disorders (NORD),[5] a national nonprofit group advocating for individuals and families affected by rare diseases.[6] Florida was the 19th state to establish a RDAC through legislation.[7]

Florida’s RDAC is directed to:[8]

- Consult with experts on rare diseases and solicit public comment to assist in developing recommendations on improving the treatment of rare diseases in Florida;
- Develop recommended strategies for academic research institutions in Florida to facilitate continued research on rare diseases;

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[1] National Organization for Rare Diseases, *Rare Disease Day: Frequently Asked Questions*. Available at https://rarediseases.org/wp-content/uploads/2019/01/RDD-FAQ-2019.pdf (last visited January 19, 2024).

[2] Department of Health, *Rare Disease Advisory Council: Legislative Report, Fiscal Year 2022-2023* (2023). Available at https://www.floridahealth.gov/provider-and-partner-resources/rdac/_documents/RDACLegislativeReport2023Final_Draft.pdf (last visited January 20, 2024).

[3] Ch. 2023-239, L.O.F., line item 539A; *See also*, Department of Health, *Agency Analysis of HB 1441* (2024). On file with the Healthcare Regulation Subcommittee.

[4] S. 381.99, F.S.

[5] National Organization for Rare Disorders (NORD). *Project RDAC Year One* (2021). Available at https://rarediseases.org/wp-content/uploads/2021/11/NRD-2200-RDAC-Year1-Highlights_FNL.pdf (last visited January 20, 2024).

[6] National Organization for Rare Disorders (NORD). *About Us*. Available at https://rarediseases.org/about-us/ (last visited January 20, 2024).

[7] *Supra*, note 2.

[8] S. 381.99(4), F.S.; *See also*, the Rare Disease Advisory Council’s 2nd Legislative Report at: https://www.floridahealth.gov/provider-and-partner-resources/rdac/_documents/RDACLegislativeReport2023Final_Draft.pdf

- Develop recommended strategies for health care providers to be informed on how to more efficiently recognize and diagnose rare diseases in order to effectively treat patients; and
- Provide input and feedback in writing to DOH, the Medicaid program, and other state agencies on matters that affect people who have been diagnosed with rare diseases.

Effect of Proposed Changes

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The Program is subject to an appropriation. The bill 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 provides an effective date of July 1, 2024.

B. SECTION DIRECTORY:

Section 1: Creates s. 381.991, F.S., relating to the Andrew John Anderson Pediatric Rare Disease Grant Program.
Section 2: 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 Andrew John Anderson Pediatric Rare Disease Grant Program is subject to appropriation.

   See *Fiscal Comments*.

B. FISCAL IMPACT ON LOCAL GOVERNMENTS:

1. Revenues:

   None.

2. Expenditures:

None.

C. DIRECT ECONOMIC IMPACT ON PRIVATE SECTOR:

Private research institutions who are eligible for the Andrew John Anderson Pediatric Rare Disease Grant Program may experience a positive fiscal impact from access to this additional funding.

D. FISCAL COMMENTS:

Andrew John Anderson Pediatric Rare Disease Grant Program

According to DOH, the $500,000 that was allocated in the 2023 General Appropriations Act to fund research grants for pediatric rare diseases is intended fund the inaugural year of the Andrew John Anderson Pediatric Rare Disease Grant Program.[9]

III. COMMENTS

A. CONSTITUTIONAL ISSUES:

1. Applicability of Municipality/County Mandates Provision:

Not applicable. The bill does not appear to affect county or municipal governments.

2. Other:

None.

B. RULE-MAKING AUTHORITY:

The bill provides sufficient rulemaking authority to implement the provisions of the bill.

C. DRAFTING ISSUES OR OTHER COMMENTS:

None.

IV. AMENDMENTS/COMMITTEE SUBSTITUTE CHANGES

On January 29, 2024, the Health Care Appropriations Subcommittee adopted an amendment and reported the bill favorably as a committee substitute. The amendment removed language related to the Telehealth Minority Maternity Care Pilot Program.

On February 22, 2024, the Health and Human Services Committee adopted an amendment and reported the bill favorable. The amendment made technical changes to the Andrew John Anderson Pediatric Rare Disease Grant Program and removed all other provisions of the bill.

This analysis is drafted to the committee substitute as passed by the Health and Human Services Committee.

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[9] *Id.*