



The Florida Senate

Local Funding Initiative Request

Fiscal Year 2019-2020

LFIR#: 2635

1. **Title of Project:** Center for Rare Disease Research

2. **Senate Sponsor:** Keith Perry

3. **Date of Submission:** 03/25/2019

4. **Project/Program Description:**

Funds research which focuses on correction of both cardiac and neurological manifestations of the diseases which cause early mortality and significant disability. Friedreich's Ataxia (FA) is an inherited disease and is caused by changes in the frataxin gene. Currently, there are no treatments available. UF's research will work to restore frataxin levels and establish high impact treatments for FA.

5. **State Agency to receive requested funds :** Department of Health

State Agency Contacted? No

6. **Amount of the Nonrecurring Request for Fiscal Year 2019-2020**

Type of Funding	Amount
Operations	850,000
Fixed Capital Outlay	
Total State Funds Requested	850,000

7. **Total Project Cost for Fiscal Year 2019-2020 (including matching funds available for this project)**

Type of Funding	Amount	Percent
Total State Funds Requested (from question #6)	850,000	100.00%
Federal	0	0.00%
State (excluding the amount of this request)	0	0.00%
Local	0	0.00%
Other	0	0.00%
Total Project Costs for Fiscal Year 2019-2020	850,000	100.0%

8. **Has this project previously received state funding?** No

Fiscal Year (yyyy-yy)	Amount		Specific Appropriation #	Vetoed
	Recurring	NonRecurring		

9. **Is future-year funding likely to be requested?** No

10. **Details on how the requested state funds will be expended**

Spending Category	Description	Amount
Administrative Costs:		
Executive Director/Project Head Salary and Benefits		



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Other Salary and Benefits		
Expense/Equipment/Travel/Supplies/Other		
Consultants/Contracted Services/Study		
Operational Costs:		
Salary and Benefits		
Expense/Equipment/Travel/Supplies/Other	\$245,000 equipment expenses for a bioreactor and liquid handling equipment; \$180,000 for non-recurring expense for animal studies.	425,000
Consultants/Contracted Services/Study	\$425,000 for study start-up and study patient support.	425,000
Fixed Capital Construction/Major Renovation:		
Construction/Renovation/Land/Planning Engineering		
Total State Funds Requested (must equal total from question #6)		850,000

11. Program Performance:

a. What is the specific purpose or goal that will be achieved by the funds requested?

Research focuses on correction of both cardiac and neurological manifestations of the diseases which cause early mortality and significant disability. Friedreich's Ataxia (FA) is an inherited disease and is caused by changes in the frataxin gene. Currently, there are no treatments available. UF's research will work to restore frataxin levels and establish high impact treatments for FA.

b. What are the activities and services that will be provided to meet the intended purpose of these funds?

Research conducted by the University of Florida Powell Gene Therapy Center.

c. What are the direct services to be provided to citizens by the appropriations project?

Increased research and expansion of Proof-of-concept studies for Friedreich's Ataxia patients.

d. Who is the target population served by this project? How many individuals are expected to be served?

Persons suffering from Friedreich's Ataxia. Greater than 800 persons are expected to be served.

e. What is the expected benefit or outcome of this project? What is the methodology by which this outcome will be measured?

Increased production and utilization of gene therapy product. Demonstration of gene therapy long-term impact on patients.

f. What are the suggested penalties that the contracting agency may consider in addition to its standard penalties for failing to meet deliverables or performance measures provided for in the contract?

Will refund appropriation per department contract.

12. The owner(s) of the facility to receive, directly or indirectly, any fixed capital outlay funding. Include the relationship between the owner(s) of the facility and the entity.

No fixed capital is requested.



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13. Requestor Contact Information:

- a. **Name:** Barry Byrne
- b. **Organization:** UF Health
- c. **E-mail Address:** bbyrne@ufl.edu
- d. **Phone Number:** (352)494-4473

14. Recipient Contact Information:

- a. **Organization:** University of Florida
- b. **County:** Alachua
- c. **Organization Type:**
 - For Profit
 - Non Profit 501(c) (3)
 - Non Profit 501(c) (4)
 - Local Entity
 - University or College
 - Other (Please specify)
- d. **Contact Name:** Barry Byrne
- e. **E-mail Address:** bbyrne@ufl.edu
- f. **Phone Number:** (352)494-4473

15. Lobbyist Contact Information

- a. **Name:** Monica Rodriquez
- b. **Firm Name:** Ballard Partners
- c. **E-mail Address:** monica@ballardfl.com
- d. **Phone Number:** (850)766-6287