



The Florida Senate

Local Funding Initiative Request

Fiscal Year 2025-2026

LFIR # 2250

1. Project Title

2. Senate Sponsor

3. Date of Request

4. Project/Program Description

The UF Health Center for Advanced Therapeutics has been established to provide and develop gene therapy treatments for rare genetic diseases. Nearly 2 million Floridians are affected by a rare genetic disease. In addition to providing specialized care to these individuals, we have developed an approach to conducting critical Investigational New Drug (IND) enabling studies to advance these therapies in clinical trials. These studies include preclinical efficacy, safety assessments, and manufacturing process optimization. Ultimately, this work will support training gene therapists, ensuring a skilled workforce capable of delivering these innovative treatments and expanding access to life-changing therapies for patients.

5. State Agency to receive requested funds

State Agency contacted?

6. Amount of the Nonrecurring Request for Fiscal Year 2025-2026

Type of Funding	Amount
Operating	750,000
Fixed Capital Outlay	0
Total State Funds Requested	750,000

7. Total Project Cost for Fiscal Year 2025-2026 (including matching funds available for this project)

Type of Funding	Amount	Percentage
Total State Funds Requested (from question #6)	750,000	20%
Matching Funds		
Federal	0	0%
State (excluding the amount of this request)	0	0%
Local	3,000,000	80%
Other	0	0%
Total Project Costs for Fiscal Year 2025-2026	3,750,000	100%

8. Has this project previously received state funding?

If yes, provide the most recent instance:

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

9. Is future-year funding likely to be requested?

a. If yes, indicate nonrecurring amount per year.

b. Describe the source of funding that can be used in lieu of state funding.



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We will pursue funding from the NIH and patient foundation grants by applying for targeted opportunities specific to each rare genetic disease under study. This includes NIH grants dedicated to gene therapy research, translational studies, and clinical trial support, ensuring sustained progress in developing innovative treatments.

Complete questions 10 and 11 for Fixed Capital Outlay Projects

10. Status of Construction

a. What is the current phase of the project?

- Planning
 Design
 Construction
 N/A

b. Is the project "shovel ready" (i.e permitted)?

No

c. What is the estimated start date of construction?

d. What is the estimated completion date of construction?

e. What funding stream will be used for ongoing operations and maintenance of the project?

11. List the owners of the facility to receive, directly or indirectly, any fixed capital outlay funding. Include the relationship between the owners of the facility and the entity.

12. Details on how the requested state funds will be expended

Spending Category	Description	Amount
Administrative Costs:		
Executive Director/Project Head Salary and Benefits	Director: Barry Byrne, MD, PhD (0.2 FTE) Associate Director: Manuela Corti, PT, PhD (0.2 FTE)	150,000
Other Salary and Benefits		0
Expense/Equipment/Travel/Supplies/Other		0
Consultants/Contracted Services/Study	Project Manager (0.5 FTE)	50,000
Operational Costs		
Salary and Benefits	Physical Therapist (0.5 FTE)	50,000
Expense/Equipment/Travel/Supplies/Other	Preclinical Studies	500,000
Consultants/Contracted Services/Study		0
Fixed Capital Construction/Major Renovation:		
Construction/Renovation/Land/Planning Engineering		0
Total State Funds Requested (must equal total from question #6)		750,000

13. Program Performance

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



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To meet the intended purpose of these funds, we will conduct critical research and development activities to advance gene therapy treatments for rare genetic diseases including Duchenne Muscle Dystrophy, Friedreich's Ataxia, Barth syndrome. This includes:

- 1) Performing IND-enabling studies, such as preclinical efficacy and safety assessments, to ensure regulatory compliance and readiness for clinical trials.
- 2) Optimizing gene therapy manufacturing processes to enhance scalability and therapeutic effectiveness.
- 3) Conducting translational research to bridge the gap between laboratory discoveries and real-world clinical applications.

These activities will accelerate the development of life-changing gene therapy treatments, ultimately improving outcomes for patient with rare genetic disorders.

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

We will create comprehensive reports for all activities conducted under this project. These reports will document IND-enabling studies, preclinical efficacy and safety assessments, gene therapy manufacturing optimization, workforce training efforts, and translational research progress. The reports will ensure transparency, regulatory compliance, and data-driven decision-making while supporting future funding applications and clinical trial readiness.

c. What direct services will be provided to citizens by the appropriation project?

The appropriation project will provide a transformative impact on public health by developing life-changing gene therapy treatments for rare genetic diseases. These therapies have the potential to cure or significantly improve the quality of life for patients who currently have limited or no treatment options. By advancing these innovative therapies through rigorous research, IND-enabling studies, and clinical development, this project will directly benefit affected individuals and families, offering hope and a path toward effective, long-term treatment solutions.

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

The target population served by this project includes individuals (children and adults) affected by rare genetic diseases who currently have limited or no treatment options. By developing life-changing gene therapy treatments, this project will provide hope and potential cures for these patients. While the exact number of individuals served will depend on the specific diseases targeted, thousands of patients nationwide could benefit from these therapies as they progress through clinical trials and into broader use.

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

The success of this project will be measured through: 1) Completion of IND-enabling studies, including preclinical efficacy and safety assessments; 2) Regulatory milestones achieved, such as successful Investigational New Drug (IND) submissions to the FDA; 3) Advancement to clinical trials, indicating the readiness of gene therapies for patient testing; 4) Publication of research findings and reports, demonstrating scientific progress and transparency.

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?

The agency may consider withholding of funds until specific milestones, such as IND-enabling study completion or regulatory submissions, are met.

14. Is this project related to mitigation, response, or recovery from a natural disaster? No

a. If Yes, what phase best describes the project?

- Mitigation (reducing or eliminating potential loss of life or property)
- Response (addressing the immediate and short-term effects of a natural disaster)
- Recovery (assisting communities return to normal operations, including rebuilding damaged infrastructure)

b. Name of the natural disaster (or Executive Order # for events not under a federal declaration):



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15. Has the entity applied for or received federal assistance for this project?

- Yes, Applied
- Yes, Received
- No
- No, but intends to apply

a. If yes, provide the FEMA project worksheet ID#:

b. Provide the total project cost listed on the FEMA project worksheet:

16. Has the entity applied for or received state assistance for this project (other than this request)?

- Yes, Applied
- Yes, Received
- No
- No, but intends to apply

a. If yes, specify the program and state agency (ex. Local Government Emergency Bridge Loan, Department of Commerce):

17. Requester Contact Information

a. First Name **Last Name**

b. Organization

c. E-mail Address

d. Phone Number **Ext.**

18. Recipient Contact Information

a. Organization

b. Municipality and County

c. Organization Type

- For Profit Entity
- Non Profit 501(c)(3)



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- Non Profit 501(c)(4)
- Local Entity
- University or College
- Other (please specify)

d. First Name Last Name

e. E-mail Address

f. Phone Number Ext.

19. Lobbyist Contact Information

a. Name

b. Firm Name

c. E-mail Address

d. Phone Number

The information provided will be posted to the Florida Senate website for public viewing if sponsored by a Senator.