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FSU NEWS

FSU Institute for Pediatric Rare Diseases gets \$5M in funding at inaugural symposium



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Florida State University's Institute for Pediatric Rare Diseases – which is still in its initial stages as the state's first-of-its-kind center – has received its second major funding of \$5 million.

The check was presented Monday morning during FSU's inaugural Pediatric Rare Disease Symposium at the College of Medicine, kicking off Discovery Days, a week-long celebration of FSU research, creativity and discovery enterprise on campus.

“As we build research at FSU and our College of Medicine through our FSU Health initiative, this is a critical beachhead for us to be working on something that will literally transform the world for families and children in the future,” FSU President Richard McCullough told the Tallahassee Democrat at the event.

The pediatric institute was launched in February and is an initiative personal to state Rep. Adam Anderson, who lost his 4-year-old son Andrew to a rare genetic disorder called Tay-Sachs disease.

Anderson, a Palm Harbor Republican, approached university leadership last year to discuss forming the institute, and a \$1 million grant for initial funding helped to kickstart it.

“I firmly believe that genetics is the backbone of the future of medicine,” Anderson said during his remarks Monday. “This new consortium, today's symposium and the great work being conducted here at FSU will play a critical role in ushering in the future of medicine to the state of Florida.”

One of the university's goals for the institute is to build up research in the FSU College of Medicine by using data to practice precision medicine. This means purchasing high-throughput sequencing (HTS) technologies, which are machines that have the ability to sequence hundreds of millions of DNA molecules at a time to determine the most effective treatment for different individuals.

While the \$5 million fund was presented during the Monday event, it comes after the amount was approved by the Florida legislature a few months ago to be allocated to FSU as part of the \$116.5 billion state budget for 2024-2025.

Dr. Alma Littles, dean of FSU's College of Medicine, explained how the path to diagnosing and treating rare diseases – which affects millions of children worldwide – is often “long, complex and uncertain.”

“At FSU, we are dedicated to changing that narrative,” Littles said. “Our mission is simple, yet powerful: to bring hope to children and families by driving discoveries that can lead to new therapies and interventions for rare diseases.”

‘It's just a matter of time’

Besides the presentation of the \$5 million funding check, the university's inaugural event Monday was made up of two symposiums, one on rare disease centers and programs and the other on genomics and gene therapy.

But before the symposiums, Dr. Terence Flotte – executive deputy chancellor and dean of the University of Massachusetts Chan Medical School – delivered a keynote address followed by a Q&A session moderated by McCullough.

“There is continual progress being made,” Flotte said when McCullough asked for his view on where he sees gene therapy evolving to in the next 10 to 20 years.

“But the trouble is that many of these diseases are progressive. So if you're a parent, you may want something that can be done now, but we're not quite able to turn on a dime like that unless something was very close to the clinic to do things in any way that has a realistic chance of helping.”

To contribute to the need for more work on gene therapy, a genetic counseling master's degree program is in the process of getting approved at FSU and is expected to accept its first

students in fall 2026 after accreditation, according to the Institute for Pediatric Rare Diseases Director Pradeep Bhide.

In addition, FSU's Office of Research funded a \$1 million genetic sequencing machine for the institute to conduct studies.

"All the way from the president's office, everybody is very excited and supportive as they stand behind the institute," said Bhide, who is also a professor at the College of Medicine.

In the future, the College of Medicine hopes to establish a pediatrics clinic in Tallahassee through the institute as part of additional initiatives on the horizon.

"I know it's just a matter of time before we're celebrating a child whose life was saved by the good work that's being done here at FSU," Anderson said.

For more information

Throughout the week, FSU's Discovery Days will have more than 25 events showcasing the variety of work done across the university.

A full schedule can be found at discoverydays.fsu.edu.

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