

# Targeting the Toughest Diseases

## Episode 3 Targeting Genetic Kidney Disease

Miami Arena. March 29th, 1996.

The Miami Heat are facing the Washington Bullets.

All night long, Alonzo Mourning of the Heat has been guarded by a giant of a man named Gheorghe Muresan.

At seven-foot-<sup>11</sup>/<sub>2</sub>" tall, Muresan is the tallest player in the league with a non-stop motor.

But Alonzo Mourning is one of the greatest ever to step on the court.

When the final buzzer goes, the Heat have won 112 to 93 and Mourning has scored an incredible 50 points.

That game was a career high for Mourning. He would go on to be a seven-time All-Star, and a gold medalist in the 2000 Olympics.

But little did he know his toughest opponent was yet to come.

After returning from the Olympics in Sydney, Mourning started into his usual off-season training.

But he noticed something was wrong.

*I was experiencing lethargy, edema in my legs, swelling in my lower extremities, extremely tired, worn out.*

He thought it was jet lag. Maybe the flu. He figured he'd just take a couple days off, then resume his training.

His doctor had a different idea. He suggested they run some tests.

*I answered the phone next to the bed. And he said, 'You know, I got your results back.' He said that 'You've got this rare genetic disorder called focal segmental glomerulosclerosis.' And I said, 'Doc, what is that?' He said, 'It's a disease that scars the filters in the kidney.'*

Focal segmental glomerulosclerosis or FSGS causes scar tissue to develop on the small parts of the kidneys that filter waste from the blood.









*Part of the reason why I'm actually so excited about what we're doing here at Vertex is because we have found small molecule therapies that are investigational to the underlying cause of what I've described as APOL1-mediated kidney disease. We have evaluated these potential therapies in experimental settings and first-in-human studies, as*

And today, he's partnering with Vertex as a spokesperson, using his profile and his experience to advocate and educate.