Therapeutic Backgrounder: Focal Segmental Glomerulosclerosis (FSGS)

FSGS is a progressive form of kidney disease associated with accumulation of cholesterol and lipids in the section of the kidneys that filters wastes out of the blood for excretion as urine (glomeruli). Damage to the glomeruli causes blood protein to leak into the urine rather than remaining in the bloodstream, a condition known as proteinuria. As the level of protein increases in the urine, patients develop a specific set of symptoms, known as nephrotic syndrome. In addition to the high levels of protein in the urine, symptoms of nephrotic syndrome include lower than normal levels of protein in the blood (hypoalbuminemia), swelling, especially in the legs, and high blood pressure (hypertension). The hypertension in people with FSGS can often be very difficult to treat. The level of proteinuria greatly influences FSGS outcomes, and the prognosis is poor for those with nephrotic syndrome. Around 70% of FSGS patients present with nephrotic syndrome at diagnosis, and more than 35% of FSGS patients develop end stage kidney disease within 10 years\(^1\)-\(^3\), requiring dialysis and ultimately kidney transplant to survive. Approximately 1,000 FSGS patients receive a kidney transplant annually. The economic burden of dialysis and kidney transplantation is high. The cost of dialysis per person per year in 2011 was approximately $60,000 for Medicare patients and approximately $125,000 for private pay patients, excluding the cost of drugs\(^4\). The average cost of a single kidney transplant in 2011 was approximately $260,000\(^4\).

FSGS Market

FSGS is estimated to affect between 55,000 and 133,000 people in the United States\(^1\)-\(^3\),\(^5\)-\(^6\), with more than 5,400 new cases diagnosed annually\(^6\). This is considered an underestimate due to the limited number of renal biopsies that are performed annually, which are necessary for a definitive diagnosis of FSGS. Additionally, the number of FSGS cases are rising more than any other cause of nephrotic syndrome\(^5\). FSGS is most common in adults 18-45 years, and its occurrence is 3–4 times more common in men than women\(^3\). FSGS occurs in Blacks at a rate that is 7 times higher than in Caucasians\(^3\).

Current FSGS Treatments and Limitations

At present, there are no disease-specific treatments for FSGS and there is no cure. Current therapy focuses on maintaining adequate nutrition, controlling blood pressure and serum lipids, minimizing loss of protein in the urine, and preventing complications from edema, thereby stabilizing kidney function. The most common drug therapy includes diuretics for edema, ACE inhibitors and ARBs for reduction of proteinuria, other antihypertensive agents, and lipid-lowering drugs.

References

1. Nephcare FSGS Facts
2. USRDS 2012 Atlas of End Stage Renal Disease in the United States
6. US Census Data 2013