

Long term outcome of idiopathic nephrotic syndrome and FSGS in children

To date, there is no common effective treatment or cure and there are no FDA approved drugs to treat nephrotic syndrome (NS) or FSGS. Treatment options for patients affected by these kidney conditions have not substantially changed over the last 30 years, with the mainstay of therapy being the long term use of harsh steroids. Both diseases are typically chronic and characterized by multiple relapses, with a potential for the development of steroid dependence or resistance.

While the majority of patients diagnosed with NS will respond positively to treatment, the relapse rate is extremely high, estimated to be up to 75%¹. The diagnosis of FSGS holds a more variable prognosis. Without therapy, or lack of response to therapy, the majority of FSGS patients will experience progression to kidney failure. Unresponsive children and adults diagnosed with FSGS share a similar clinical course with 50% of patients reaching end stage renal disease (ESRD) within 5 years¹. While these diseases are common in adults we are now beginning to learn of the long term outcome of diagnosis and treatment in children.

Prikhodina and colleagues evaluated the long term outcome of 60 children diagnosed with idiopathic steroid resistant nephrotic syndrome (SRNS) in Moscow. The authors presented the results of a ten year retrospective study assessing the influence of immunosuppressive treatment on these children between 1997 and 2007. The mean age of onset of disease was 8.6 years of age².

The authors found that patients who did not receive immunosuppressive therapy had greater deterioration of glomerular filtration rate (GFR) compared with children treated with immunosuppressive drugs. GFR is a measure of how effectively the kidneys are filtering waste products. Furthermore, children with a poor response to immunosuppressive treatment had the highest risk of progression to kidney failure².

In a separate study, El-Rafaey and colleagues evaluated the clinical course of 72 Egyptian children diagnosed with biopsy proven primary FSGS between 1995 and 2008. According to this retrospective study, 48 (66.7%) patients were male. In this population 14 patients (19.4%) responded initially to treatment with steroids, 54 (75%) did not initially respond to steroids and 4 (5.6%) were steroid dependent. When kidney survival was examined, the authors found that five years following



diagnosis 93% of this population maintained kidney function but when evaluated at ten years, that number was reduced to 68% ³.

These studies complement previously published research on the long term outcome of these potentially devastating diseases. Furthermore, they help to reinforce the need for evaluation of new therapies and treatments for patients that do not respond to treatment consisting of traditional therapies.

1. Fehally J, Floege J, Johnson R. Comprehensive Clinical Nephrology.3. Philadelphia: Mosby Elsevier, 2007. Print.
2. Prikhodina L, Dlin V, Turpitko O, Ignatova M. Immunosuppressive treatment and long-term outcome of idiopathic steroid-resistant nephrotic syndrome in children: a 10 year single centre experience. Presented at the World Congress of Nephrology Annual Meeting, Milan, Italy, May 22-26, 2009.
3. El-Refaey A, Sarhan A, Bakr A, Hammad A, Ragab M, El-Hoseeny F, Abd-Elrahman A. Primary focal segmental glomerulosclerosis in Egyptian children: 10-year single center experience. Presented at the World Congress of Nephrology Annual Meeting, Milan, Italy, May 22-26, 2009.

