Nephrotic Syndrome 101
Ask an Expert Webinar Series
Ask the Expert Guest Speaker

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What do the Kidneys do?

- **Kidneys** process blood to excrete wastes and extra water and convert into urine.
- Urine produced by the kidney is carried by the **ureter** to the **bladder**.
- The **urethra** carries urine from the body to the outside.
What do the Kidneys do?

Healthy, functioning kidneys are important because they:

- **Eliminate** waste products, drugs and toxins from the blood
- **Regulate:**
  - electrolyte concentrations
  - amount of fluid within the body
  - blood pressure
- Help **maintain** acid base balance
- **Produce hormones** that affect blood and bones
The kidney is composed of a million tiny units called **nephrons**.

Each nephron is composed of a glomerulus and a tubule.

**Glomeruli** are small blood vessels through which blood enters the kidney. They filter wastes and excess fluids.

**Tubules** collect the waste to form urine.
A substance that is filtered passes first through the capillary endothelium (red). Next, it passes across the glomerular basement membrane (tan). Finally, the substance passes through the filtration slits, that are found between the interdigitating foot processes of the podocyte (blue and green).
Disruption of the Glomerular Structure Leads to Nephrotic Syndrome

The Glomerular Filtration Barrier

Failure of the Filtration Barrier in Nephrotic Syndrome
The Colander Kidney

Think of the glomeruli as a colander- it keeps the good stuff in and drains the water. When you have Nephrotic Syndrome, the colander holes are too big and the good stuff gets out.
Nephrotic Syndrome

• Nephrotic Syndrome is a condition in which protein leaks from blood into the urine

• This results in:
  - Higher protein in urine
  - Lower protein in the blood

• Distinct constellation of clinical and laboratory findings

• Damage can be from an unrelated condition (diabetes, hypertension)
  - This is called Primary, or Idiopathic

• Damage can be from a kidney disorder
  - This is called Secondary
Signs and Symptoms of Nephrotic Syndrome

- **Edema**: fluid collects in dependent tissues, causing swelling
- **Proteinuria**: protein that should remain in the blood instead leaks into the urine, causing foamy urine
- **Hypoalbuminemia**: albumin levels in the blood are below normal
- **Hyperlipidemia**: high blood lipids (cholesterol, triglyceride, etc.)
- **Lipiduria**: lipids and lipoproteins spill into the urine

*Weight gain, blood clots, susceptibility to infections*
Minimal Change vs FSGS

**Minimal Change Disease**
- Characterized by normal appearance of the filtering units of the kidney under a light microscope
- Most MCD patients respond well to treatment
- Remission allows injury to the filtering units to be reversed
- Loss of kidney function is rare

**FSGS**
- Characterized by sections of scarred tissue on the kidney
- Fewer FSGS patients respond well to treatments
- Can result in kidney failure
Under a Microscope

Normal

MCD

FSGS
In Adults

• Membranous Nephropathy (MN) – most common

• Focal Segmental Glomerulosclerosis (FSGS)

• Other Forms Include
  – Minimal Change Disease (MCD) – increasing minority
  – Alport Syndrome
  – C1q Nephropathy
  – IgA Nephropathy
  – IgM Nephropathy
  – Membranoproliferative Glomerulonephritis (MPGN)

In Children

• Minimal Change Disease (MCD) - decreasing majority

• Focal Segmental Glomerulosclerosis (FSGS)

• Other Forms Include:
  – Alport Syndrome
  – C1q Nephropathy
  – Congenital Nephrotic Syndrome
  – IgA Nephropathy
  – IgM Nephropathy
  – Membranous Nephropathy (MN)
  – Membranoproliferative Glomerulonephritis (MPGN)
Seeing a Nephrologist

Checklist of things to discuss with your nephrologist:

✓ **Diagnosis** / extent of damage
  ➢ Usually a biopsy is performed on adults
✓ **Treatment options** - short and long term
✓ **Side effects** / medication **interactions**
✓ **Diet, exercise, and lifestyle changes** that should be implemented
✓ What **to do if** symptoms get worse
✓ What to do if in **an emergency** situation

**Pediatric Concerns:**

All the above, **PLUS**

✓ **Vaccinations** that may be needed
✓ **Is a biopsy necessary?**
  ➢ Biopsies are **usually not** performed on children

Always remember that you are entitled to a **second opinion**. It is important that you (and your family) feel comfortable with your doctor and your treatment plan.
Treatment Goals

Short Term

• **Reduce /eliminate protein spillage** in the urine
• **Improve** clinical symptoms (swelling, edema, fatigue)
• **Correct abnormalities** of the blood levels (cholesterol, albumin)

Long Term

• **Prevent recurrences** of protein spillage into the urine
• **Preserve kidney** function
• **Avoid** treatment-related complications
Treatment of NS

Widely accepted initial therapy for both children and adults: **Prednisone**

- If there are frequent relapses, or the patient is non-responsive,
  - Talk to your doctor about adding other medications to the prednisone
  - Talk to your doctor about trying a different therapy plan
  - Be prepared for the “laundry list” of side effects that may occur
  - Talk to your doctor about clinical trials

Prednisone acts as an immunosuppressant. This will help reduce swelling, and, hopefully, help the kidneys function properly.
Other Treatment Options

Primary Nephrotic Syndrome & the related diseases are difficult to treat because of the variable clinical courses.

• There are many off-label therapies that you can talk to your doctor about as a second-line treatment option.

• Clinical trials are always an option
  – For both children and adults.

• Some patients have found complimentary therapies useful
  – Acupuncture, various supplements, diet and lifestyle changes.

Common Second-Line Therapies

- Abatacept
- ACE/ARB
- Acthar
- Cyclophosphamide (Cytoxan)
- Cyclosporine
- Tacrolimus (Prograf)
- Mycophenolate (Cellcept or Myfortic)
- Rituximab
PROGNOSIS

• Many factors influence the long-term outcomes, including:
  - **Gender**
  - **Severity** of kidney dysfunction
  - **Frequency** of relapses
  - **Responsiveness** to treatment
  - **Degree** of proteinuria
  - **Age** of onset

• **What we do know**
  - Pediatric transplants are relatively rare
  - MCD patients respond relatively well to initial therapies
  - For many glomerular diseases, remission may take longer (up to 2 years) to reach
SUMMARY

- Nephrotic Syndrome is characterized by protein in the urine
  - Signs= fatigue, swelling, weight gain, foamy urine
  - It **signifies** that there is damage to the kidney / the kidneys are not properly working
- Treatment goal = reduce proteinuria, **lower** blood pressure, **preserve** kidney function
- Each therapy has its own risks and side effects- have an **open and honest conversation** with your doctor
- Lifestyle **changes** help reach treatment goals
- Prognosis can **vary depending** on each patient’s situation
FAQs About Nephrotic Syndrome

• Do I need another biopsy?

• Does my child need a biopsy? Why?

• What does it mean for me or my child to be steroid resistant or steroid dependent?

• What is the most efficient way to monitor symptoms at home?

• How important are dietary restrictions in controlling my NS?
Questions?
Thank you for attending!!!

Plan on joining our next Ask the Expert webinar on February 18th
I’m Ready to Participate in Research...Now What?