Finding The Best Care for Kids With Lupus

Pediatric Rheumatologists are doctors who treat autoimmune and muscle, bone, and joint diseases. Lupus is an autoimmune disease that can affect joints, skin, brain, lungs, and other body parts. Autoimmune means instead of protecting us, the immune system hurts us. These doctors are working to standardize care for kids with lupus. This means that each child in every clinic who has the same problem will get the same care.

Standardized care lets researchers (people who study a problem) compare different medicines, find what works best, and make sure all kids get the best care possible. Complications from lupus can lead to severe illness or death. That is why it is crucial that we find the best way to treat it.

The Food and Drug Administration (FDA) approves new drugs and spells out how they should be used. Right now there are no FDA-approved treatments for kidney disease from lupus, and no clear-cut studies to help doctors choose care.

In the past, care was guided by large research studies called randomized controlled trials. In this type of study, many similar patients are put into at least 2 different groups, given different care based on their group, and watched over time to study what happens. These studies are costly, slow, and do not work well for rare diseases like childhood lupus.

Another research style, called comparative effectiveness research, compares at least 2 kinds of care doctors normally give their own patients. It needs a reliable way to collect data on how the patient is doing. This is why the Childhood Arthritis and Rheumatology Research Alliance (CARRA) set up a registry (list of patient data). Patients sign up in their doctor’s office, fill out some paper questionnaires, and agree to add their data to the registry.

Comparative effectiveness research requires doctors to treat patients in only a few different ways. Right now doctors treat children with lupus in many different ways. To solve this problem, Pediatric Rheumatologists from CARRA agreed on a few specific care plans using previous research and their shared knowledge.

The first step was to decide on the best ways to look at different stages of care: 
- induction therapy (getting the disease under control) and 
- maintenance therapy (avoiding flares when medicines are lowered after the disease is under control)

Researchers asked doctors across the U.S. what treatments they were using. They learned there were too many different therapies in use to make a comparison.
Researchers then used discussion groups, surveys, and published reports to narrow down care choices. At the end, they chose the following medicines that calm the immune system when it starts to hurt us:

- 2 options for induction therapy
  - cyclophosphamide (also called cytoxan)
  - cellcept (also called mycophenolate mofetil)
- 3 options for maintenance therapy
  - continued cyclophosphamide
  - cellcept
  - azathioprine (also called imuran)

In addition to these medicines, people with kidney disease need steroids. People joke that there are as many different steroid doses as there are rheumatologists. But these researchers were able to agree on 3 steroid dosing levels, plus a standard way to slowly reduce the steroid dose. This matters because it is not safe to stop steroids quickly, and there are many different ways to reduce the dose.

Researchers then wondered if doctors would really use these treatment plans, and how patients would do. They got funding from the Lupus Foundation of America and the Arthritis Foundation to test the treatments and collect data at a few sites.

So far, 40 patients at 10 sites have been studied. Researchers are learning when the treatment plans are being started and how long they are used. If doctors are not giving the treatments as planned, the treatments may need to be changed.

Next, researchers will seek funding to study a larger group of patients. When enough patients have used the treatment plans and added their data to the registry, doctors hope to know which treatments work best, and which have the least side effects. Until then, standardized treatment plans can help doctors who have less experience caring for children with lupus.

Little is known about the autoimmune disease Systemic Lupus Erythematosus (SLE). About 1 in 7 cases start during childhood, and the illness can be worse when it occurs in young people. Nephritis (lupus in the kidneys) affects over half of children with SLE, and can lead to kidney failure, dialysis or kidney transplant, and sometimes death. Thus it is crucial that we find the best way to treat it.

This article was written by the patients, families, researchers and member organizations of PARTNERS.