

## GRAND ROUNDS

April 16

### Novel Therapies for Neonatal Pulmonary Hypertension

Judy Aschner, M.D., professor of Pediatrics, Vanderbilt University Medical Center; Director of Neonatology, The Monroe Carell Jr. Children's Hospital at Vanderbilt, Nashville, TN.

April 23

### From Jenner to Genome: New Approaches to Vaccinology

Kathryn M. Edwards, M.D., Vice Chair, Pediatric Clinical Research Office, Vanderbilt University Medical Center, Nashville, TN.

April 30

### Severe Combined

### Immunodeficiency: A Pediatric Emergency

Maria de la Morena, M.D., associate professor of Pediatrics, Division of Allergy and Immunology, UT Southwestern.

*Also available via videoconference at selected area hospitals, call 214-345-2330 for locations.*

## MEDICAL UPDATE: Prednisone and Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is the most common type of muscular dystrophy in childhood. There are approximately 180 patients between two and 18 years of age followed in the Pediatric Neuromuscular Clinic at Children's. Affected boys stop walking by age 10 years and may die of respiratory or cardiac failure in the second or third decade. However, new management protocols have improved the outlook for these patients. A recent review documented improvement in survival for patients diagnosed with DMD after 1990. Use of non-invasive ventilation and prednisone mean it is not uncommon for patients with DMD to reach the age of 30, graduate college or work full time.

In the past decade, treatment with prednisone has become standard of care based on clinical trials performed between 1986 and 1995. The recommended dose is 0.75 mg/kg/day. Alternative dosage schedules are currently under study. It was established early that every other day prednisone did not give the same benefit as daily dosing. For many patients the benefits are worth the risk. On average, boys treated with

steroids walk two years longer than their peers, maintain normal lung function through adolescence and avoid scoliosis surgery. Long-term benefits have been documented in a cohort of DMD patients who were treated for 12 years or more. Most neuromuscular specialists recommend beginning prednisone around age five and continuing life-long. Many patients avoid the severe side effects of prednisone by eating a healthy diet (low sodium, low carbohydrate) and maintaining a structured life-style including regular exercise and sleep. If side effects are not tolerated, the dose can be lowered while maintaining some benefit.

The mechanism whereby prednisone slows the progression of disease is unknown, but it is not an anti-inflammatory action. Basic investigation is underway to identify other drugs that may slow the progression of DMD without causing the side effects of prednisone.

For more information on prednisone treatment, contact Susan Iannaccone, M.D. at 214-456-5220 or [susan.iannaccone@utsouthwestern.edu](mailto:susan.iannaccone@utsouthwestern.edu)

## SERVICE UPDATE: Children's pediatric motility service is first academically affiliated center of excellence in North Texas

Children's is the first academically affiliated hospital in North Texas to support a pediatric GI Motility center of excellence. The Children's Pediatric GI Motility Center, one of a handful in the U.S., began operations last fall as a subspecialty service within GI and encompasses centers for bowel and aerodigestive disorders management. Patients are seen at the Children's Dallas Ambulatory Care Pavilion. Medical staff at the center diagnose and treat severe intractable constipation, functional abdominal pain and swallowing difficulties using various manometry procedures, state-of-the-art high resolution manometry and advanced impedance measurements. Children's is the only academically affiliated pediatric facility in North Texas to employ this technology.

For more information about the Children's Pediatric GI Motility Center and services offered, call 800-CHILD-RX (244-5379).

CHILDREN'S  
MEDICAL CENTER

Physician Referrals  
**800-CHILD-RX**

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