



Research Now. | **JANUARY 2015**

*The Research Institute at Nationwide Children's Hospital*



*Muscle biopsy from a lab in the Center for Gene Therapy. Analysis of these samples helped assess the efficacy of gene therapy for patients with Becker muscular dystrophy.*

## RESEARCH AND DISCOVERY

### [Gene Therapy Trial Demonstrates Improved Walking Performance over Placebo for Patients with Becker Muscular Dystrophy](#)

Jerry Mendell, MD, director of the Center for Gene Therapy, and Brian Kaspar, PhD, principal investigator in the Center, is co-author of a recent publication on the first human clinical trial involving follistatin gene therapy for patients with Becker muscular dystrophy.

### [Knowledge and Attitudes About Genetic Testing Held by Parents of Children with Congenital Heart Defects](#)

Sara Fitzgerald-Butt, MS, LGC, genetic counselor in the Center for Cardiovascular and Pulmonary Research and The Heart Center, collaborated on a study examining genetic knowledge and attitudes of parents of children with congenital heart defects, since genetic testing for specific defects is becoming standard of care in pediatric cardiology.

### [Infection Susceptibility Depends on Kidney pH Balance and Key Enzyme](#)

Andrew Schwaderer, MD, research director of Nephrology, is senior author of a study conducted by clinician-scientists at Nationwide Children's Hospital and collaborating institutions, which evaluated the role of a key enzyme involved in improving the body's ability to defend itself without antibiotics.

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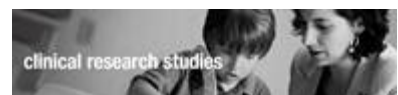
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## RECENT NEWS

[Department of Pediatrics Recognizes Outstanding Faculty for 2014](#)

[Nationwide Children's Hospital Participating in PEDSnet, National Pediatric Learning Health System](#)

[Safety Coach Program Expands to Research](#)

## MEETINGS AND EVENTS

[Next Research Live Show is January 28 at Noon](#)

[State of the Research Institute Address on January 28 at 3PM](#)

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## RECENT MEDIA COVERAGE

[Patent on Experimental Gene Therapy Designed to Reverse Muscular Dystrophy](#): The patent is assigned to Nationwide Children's Hospital and licensed to Milo Biotechnology, and covers a therapy that was developed by researchers at Nationwide Children's (Crain's Cleveland)

[Toys Send Kids to the ER Every Three Minutes](#): Gary Smith, director of the Center for Injury Research and Policy, is lead author of a recent study in *Clinical Pediatrics* that found an increase in toy-related injuries over recent decades (Forbes)

[\\$3.6M Raised to Develop Therapies for Children with Sanfilippo Syndrome](#): Joint efforts of Abeona Therapeutics, supporting Sanfilippo foundations, and researchers at Nationwide Children's Hospital aim to accelerate development of therapies (Biospace)

[Flu Vaccine Fighting a Moving Target](#): Mark Peeples, PhD, a principal investigator in the Center for Vaccines and Immunity, discusses challenges associated with developing a universal flu vaccine (Columbus Dispatch)



[2014 Fall/Winter Issue of Pediatrics Nationwide](#)