

# Children's Health Update

## Basic Research Leads To New Treatments

(NAPSA)—Funding for basic medical research is paying dividends when it comes to battling many diseases. In one instance, it has helped to develop a treatment for a condition that ages children before their time...and may hold clues to aging-related heart disease.

Hutchinson-Gilford Progeria Syndrome (HGPS)—progeria, as it's more commonly called—has been described as out of control, rapid aging in children. In the past, progeria was simply too rare to attract much scientific attention. Approaches were limited to physical and occupational therapy, special nutrition and adult-based strategies to control related heart and circulatory disease.

Recently, however, a possible treatment for this “untreatable” disorder has emerged from basic cell biology, the Human Genome Project, and a new use for a “failed” cancer drug. The Human Genome Project was funded by the National Institutes of Health (NIH) and taxpayers' dollars.

The progeria bench-to-bedside story has unfolded with breathtaking speed: only five years from discovering the progeria gene to a potential treatment for these children. Experts say this may be the fastest example of turning basic research into clinical application.

By funding decades of “nontargeted” basic research involving cells, for example, at universities throughout the country, the NIH has made this breakthrough possible. In addition, a nonprofit “disease” foundation, organized by parents of children with progeria, also played a role.

A clinical trial began last spring in Boston and will continue for two and a half years. Clinical trials are required to test how effective a drug or treatment is in



Photo courtesy of The Progeria Research Foundation

**Lindsay, 4 years old, may benefit from a new treatment developed by NIH-funded cell biologists.**

people before the Food & Drug Administration (FDA) will consider approval. The outcome of this trial may be a matter of life and death for many of the 28 HGPS children enrolled in this trial, since their average life span is currently 13 years.

However, there are implications beyond this tiny population. Researchers say that a treatment for HGPS kids may open a whole new perspective on “normal” aging and its complications, particularly in cardiovascular disease.

The progeria story is but one example of how unexpected discoveries in basic research can lead to unsuspected connections in human health. Finding the progeria gene would have been hopeless without the grand-scale Human Genome Project or a “failed” cancer trial that provided researchers with the necessary foundation for their promising work.

With more links between basic research and clinical results, new treatments for other diseases could be just around the corner.

To learn more, visit [www.ascb.org/progeria](http://www.ascb.org/progeria).