# No longer waiting idly: Parents of patients establishing gene therapy programs

In recent years, gene therapies have become safer and better at hitting their intended targets in the body, leading to a handful of remarkable cures in clinical trials. Advocates for rare-disease patients—especially determined parents—are increasingly seeking to start gene-therapy programs. They are establishing patient advocacy organizations, raising money for research, and even founding their own biotechnology startups to find treatments where few or none currently exist.

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“The prospect of being able to correct these genetic abnormalities with gene therapy has become a topic of great interest in the rare-disease community,” says Mary Dunkle, vice president of educational initiatives at the National Organization for Rare Diseases.

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Historically, much of the research on rare diseases has been driven by patients and patient organizations that have raised funds for grants and reached out to medical researchers, says Dunkle. “It’s not surprising that parents of children with devastating diseases would be doing whatever they could to try to save the lives of their children,” she adds. “For them, the clock is ticking, and there is a powerful sense of urgency.”

**The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post:** Determined Parents are Moving the Needle on Gene Therapy

**For more background on the Genetic Literacy Project, read GLP on Wikipedia.**