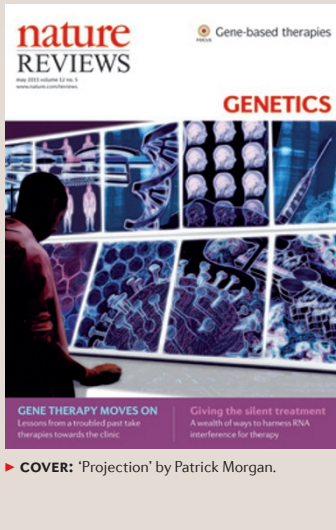




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This issue of *Nature Reviews Genetics* features a Focus on Gene-Based Therapies. The past few years have seen renewed excitement in this field, with many gene-based therapies now in, or nearing, clinical trials.

After intense enthusiasm at the inception of this field, researchers ran into serious problems — including safety concerns — that slowed progress. In his Review on p316, Mark Kay discusses how many hurdles to gene-based therapy have been tackled by improvements to vectors.

The 'traditional' mode of gene-based therapy is the delivery of a therapeutic transgene using viruses that target certain tissues and cell types. The greatest progress with this type of therapy has been achieved using adeno-associated virus vectors — the topic of the Review from Federico Mingozzi and Katherine High on p341. The authors highlight the fact that each tissue type has its own set of challenges as a therapeutic target.

An area that shows great therapeutic promise is the combination of cell-based delivery with gene-transfer technologies. Luigi Naldini explores the wide range of applications of this mode of therapy, which is being used to tackle diseases from immunodeficiencies to cancer (Review, p301). Approaches for monitoring and reducing insertional mutagenesis have been important developments in this field.

Finally, much enthusiasm has surrounded the harnessing of RNA interference (RNAi) for therapeutic use, which is the topic of the Review on p329. Beverly Davidson and Paul McCray discuss the diverse approaches that can be used to develop RNAi for therapy and the progress in taking these therapies to clinical trials.

The Focus is free to all readers for one month at: <http://www.nature.com/nrg/focus/genetherapies>.



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