900 gene therapy drugs are in the pipeline. How does the FDA want to regulate them?

To date, the FDA has approved four gene therapy products, which insert new genetic material into a patient's cells. The agency anticipates many more approvals in the coming years, as evidenced by the more than 900 investigational new drug (IND) applications for ongoing clinical studies in this area. The FDA believes this will provide patients and providers with increased therapeutic choices.

In that spirit, [January 28], the FDA is announcing the release of a number of important policies: six final guidances on gene therapy manufacturing and clinical development of products and a draft guidance, Interpreting Sameness of Gene Therapy Products Under the Orphan Drug Regulations.

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The six guidance documents incorporate input from many stakeholders and take a significant step toward helping to shape the modern structure for the development and manufacture of gene therapies.

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In sum, these policy documents are representative of efforts to help advance product development in the field of gene therapy. We will continue to work with product innovators, sponsors, researchers, patients, and other stakeholders to help make the development and review of these products more efficient, while putting in place the regulatory controls needed to ensure that the resulting therapies are both safe and effective.

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