Gene therapy boost: FDA positions for faster reviews of new treatments

The Food and Drug Administration on [November 16] issued new guidelines to speed the introduction of treatments involving human cells and tissues, including gene therapy. But the agency also said it would crack down on rogue clinics offering dangerous or unproven versions of those treatments.

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These concepts are no longer the stuff of science fiction," said Scott Gottlieb, the commissioner of the F.D.A., "but rather, real-life science where cells and tissues can be engineered to grow healthy, functional organs to replace diseased ones; where new genes can be introduced into the body to combat disease; and where adult stem cells can generate replacements for cells that are lost to injury or illness."

Gene and cell therapies that demonstrate the potential to treat unmet medical needs and serious illnesses may now qualify for expedited review to get the products to market more quickly, the guidelines say. The F.D.A. will still require clinical trials, but it is promising a faster process, as required by Congress under the 2016 21st Century Cures Act.

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So far, the drug agency has approved only two products that qualify as gene therapy — Kymriah, from Novartis, and Yescarta, made by Kite Pharma. Both treatments involve genetically altering a patient's own immune cells to fight leukemia or lymphoma.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: F.D.A. Speeds Review of Gene Therapies, Vowing to Target Rogue Clinics