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# FDA FINALIZING POLICIES TO PROMOTE GENE THERAPIES

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FDA will soon release a framework to facilitate manufacturing of gene therapies, and will lay out development strategies for gene therapies to treat specific diseases, FDA Commissioner Scott Gottlieb said Tuesday.

In remarks prepared for delivery at a meeting of the Alliance for Regenerative Medicine, Gottlieb highlighted the need to overcome manufacturing challenges, as well as the potential role of accelerated approval to speed therapies to patients while ensuring that long-term efficacy and safety questions are answered.

Guidance concerning hemophilia treatments will be among a suite of gene therapy guidance documents FDA will release as part of its framework, Gottlieb reported. “The first therapeutic area we’ll focus on is hemophilia, where factor production may be sufficient in some cases as a surrogate measure of benefit where a gene therapy product can potentially normalize factor production,” he said.

Gottlieb added that “demonstration of a reduction in bleeding rates could be confirmed post approval, as we continue to study a product’s long-term safety and durability.”

The biggest challenges to widespread adoption of both cellular and gene therapies are limitations on manufacturing capabilities, Gottlieb said. To address these challenges, FDA is working to help industry improve manufacturing, and is encouraging sponsors to change the way they think about transitioning from clinical trials to commercial-scale production.

Gottlieb highlighted an initiative FDA is undertaking “internally, and in collaboration with a variety of different partners, to help improve the yield of cell lines used to produce gene therapy vectors.” He noted that the agency is also investigating the application of continuous manufacturing technologies to the production of gene and cell therapies.

The commissioner also called on sponsors of gene therapies to move away from the drug development scale-up paradigm in which small quantities of a new molecule are produced in a pilot plant for early stage trials, and if trials are successful, there is a transition to commercial-scale manufacturing.

For gene therapies, transitioning from a pilot to a commercial process can "significantly delay access or even lead to abandonment of development, as the manufacturing transition can be costly and challenging,” he said. As an alternative, FDA is “encouraging sponsors, particularly those working with small populations, to consider developing scalable manufacturing processes with inherent quality attributes that can potentially support scale up and licensure."

Gene therapy stocks reacted variably to the news, with BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) losing $1.75 to $87.61. Spark Therapeutics Inc. (NASDAQ:ONCE) gained $1.74 to $74.83 and uniQure N.V. (NASDAQ:QURE) added $0.84 to $32.34.