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US FDA's Califf: We Don't Need Much Help From Congress On Rare Diseases

by Derrick Gingery

The FDA commissioner also does not want the agency setting goals for rare disease treatment development.

Existing tools are more than enough for the US Food and Drug Administration to combat rare disease, but issues outside its purview may require more help, Commissioner Robert Califf said.

During a 29 February appearance on Washington Post Live to discuss rare disease issues, Califf said the agency cannot significantly impact issues, such as funding research and paying for the treatments that are produced, but they may be areas where Congress could act.

"This may be one of the rare times where you'll hear me say that we don't need that much from Congress at this point," he said. "We can always tweak the system, but we have tremendous tools at our disposal as it relates to FDA."

Califf made the comments as many rare disease patients and advocates are in Washington D.C. lobbying for legislation intended to make treatments available sooner or more affordable.

Califf would not comment when asked during the event about the Promising Pathway Act, which would create a provisional approval mechanism for

Accelerated Approval Now Starting Point For Gene Therapy Development, US FDA's Marks Says

By Derrick Gingery

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Expedited approval pathway had been a secondary option when traditional approval was not possible, but the CBER director wants surrogate endpoints increasingly considered from the beginning, especially with pediatric rare disease gene therapies.

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products that could not access accelerated approval. But he said the problems need creative solutions and the FDA was open and interested in the discussion.

The conditional approval proposal is partially intended to ensure the FDA uses its regulatory flexibility more consistently, but some FDA officials are not sure another approval pathway is necessary to expedite rare disease approvals. (Also see "*Promising Pathway Act Would Fill Gap For Rare Disease Treatments That Don't Fit Accelerated Approval, Advocates Say*" - Pink Sheet, 26 Oct, 2023.)

Califf also said policymakers need to be creative to ensure the affordability and postmarketing follow-up requirements for cell and gene therapies, which are growing in popularity as rare disease treatments.

Follow-up can last 15 years, but sponsors must ensure postmarketing studies of gene therapy patients are completed. (Also see "*US FDA Struggling With Long-Term Follow-Up Requirements For Gene Therapies*" - Pink Sheet, 18 Oct, 2023.)

Several ideas also have been floated for easing the payment burden for the sometimes multi-million-dollar gene therapy prices. (Also see "*Medicaid Payments For Cell, Gene Therapies Outside Hospital Bundle Supported By Developers*" - Pink Sheet, 1 Feb, 2024.)

FDA Should Not Set Rare Disease Approval Goals, Califf Said

Califf also said the FDA should not be setting goals for the number of rare disease treatments that should be developed or approved.

"I feel like it's probably not right for us to set that target," he said. "Ultimately, it's an ecosystem where we all need to work together."

With more than 10,000 known rare diseases and only a fraction of them with approved treatments, stakeholders have been pushing to ramp up the speed of development and approval.

"Rather than set a goal, I would say we've got to be making measurable progress," Califf said. "We should see an acceleration of that progress, not just a linear trend, but more of an exponential trend, because of the beauty of the science now is lending itself to platforms that might be useful across a whole

IRA Innovation Debate Showcased At House Hearing On Rare Disease Legislation

By Cathy Kelly

29 Feb 2024
Bills to narrow the Medicare price negotiation program's reach generated extensive discussion at the hearing.

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variety of diseases."

The 2022 Food and Drug Omnibus Reform Act (FDORA) created the platform technology designation, which could allow multiple gene therapies using the same AAV backbone on the market faster.

Center for Biologics Evaluation and Research Director Peter Marks said 26 February during the Biopharma Congress that no designations have been granted yet, but sponsors are interested in the program.

Platforms could improve the gene therapy business model and encourage more development. (Also see "*'Pull Every Lever': FDA's Marks Worries Gene Therapies Could Falter Without Help To Lower Cost*" - Pink Sheet, 13 Nov, 2023.)