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Rare Cancer Development Consortium Moving Toward Target Selection

by Derrick Gingery

An upcoming public meeting will include a look at platforms before the first targets are chosen.

National Cancer Institute officials will select the first targets for the US Food and Drug Administration-initiated open-source oncology development program. Stakeholders and others are expected to be more involved in later rounds.

Project Catalyst, which was developed by the FDA's Oncology Center of Excellence, has been working to entice academia, industry, advocates and others to create a group that would develop treatments for rare tumors not viable for commercial development.

The program is progressing to a stage where drug development will begin. A public meeting in late spring or early summer will include a look at potential platforms that could target tumor vulnerabilities.

Jeff Summers, associate director for translational sciences in the FDA's Center for Drug Evaluation and Research Office of Oncologic Diseases, said that while he wants the oncology community to contribute, NCI is going to determine the consortium's direction at first.

"We really want to include the community and we don't want to alienate anybody, but we really also have to focus," he said during the FDA Rare Disease Day conference in March. "There are a lot of ultra-rare tumors that just have targets that have been there for 30 years that have been just begging to be targeted."

Summers said the consortium will solicit ideas from the rare disease community after the first round is completed, which should take a year or two.

"We want the community to help us understand that science, why particular targets or the

science of the disease might avail itself to some particular platforms and then we would consider those," he said. "It will be an interactive process, but the first iteration going forward will be a little more directed from the National Cancer Institute."

Work by the consortium is expected to be done in an open science and transparent manner with no intellectual property claims. (Also see "*US FDA Exploring Consortium To Develop Treatments For 'Economically Infeasible' Cancer Indications*" - Pink Sheet, 3 Apr, 2023.)

Project Catalyst was created by OCE to provide educational resources and answer regulatory science questions for small companies and others with little or no experience moving an oncology application through the FDA. (Also see "*US FDA Project Protect Oncology Safety Program In Broad Use After Quiet Launch*" - Pink Sheet, 28 Mar, 2022.)

Consortium Would Publish Meeting Minutes, Protocols, Study Data

Stakeholders discussed creating a public-private partnership (PPP) to support the idea during an August 2023 public meeting.

A PPP can use government and private sector resources to de-risk development in the ultra-rare space, as well as "establish a dynamic process that can be refined and reutilized," speakers said during the meeting, according to a *summary*.

FDA staff are expected to be liaisons that provide "strategic, regulatory and scientific insights," including on target selection and chemistry, manufacturing and controls, during product development, according to the summary.

Liaisons would not be included on application assessment teams that would make regulatory decisions.

As part of the "open notebook" approach to development, minutes and notes from scientific advisory board, steering committee and other meetings will be made public, along with study protocols and results. Information about challenges and course corrections also would be released, according to the summary.

Summers said during the Rare Disease Day event that the FDA was impressed with the commitment of the partners involved.

"The idea is for others who are interested in ultra-rare cancer drug development to learn from our challenges," he said. "In my opinion, the solution to any problem is already out there and that's you the community."

After the meeting to evaluate data and select targets, PPP governance will be developed and pilot

partnerships for drug development formed.

Growing List Of Public-Private Partnerships In Therapy Development

A rare tumor PPP likely would follow in the mold of similar groups, like the Bespoke Gene Therapy Consortium, which is working to streamline the manufacturing of gene therapies for rare and other diseases that are not commercially viable.

Ideally, the consortium would provide small batch manufacturing and vector generation facilities and help create a low-cost, repeatable process. (Also see "*Gene Therapy Manufacturing Hurdle: Sponsors Unwilling To Share 'Secret Sauce'*" - Pink Sheet, 10 Aug, 2020.)

The consortium released its first "playbook" in February, which described the pre-IND process for those who are not familiar with the development process. (Also see "*Bespoke Gene Therapy 'Playbook' Outlines Platform Approach To AAV-Based Treatments*" - Pink Sheet, 15 Feb, 2024.)