FDA's New ARC Program Centralizes and Accelerates Rare Disease Activities

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Last week, the Center for Drug Evaluation and Research (CDER) at the Food and Drug Administration (FDA) launched a new program to coordinate and centralize CDER's rare disease activities. The mission of the Accelerating Rare disease Cures (ARC) Program is to drive "scientific and regulatory innovation and engagement to accelerate the availability of treatments for patients with rare diseases." As CDER Director Patrizia Cavazzoni has stated, the newly established ARC program will act as "connective tissue" for enhancing and coordinating the rare disease activities across the Center, without changing its organizational structure.

Over the years, rare disease drug approvals have increased substantially -- orphan drugs comprised 52% of CDER's novel drug approvals in 2021. Despite this progress, significant unmet need remains. By some estimates, over 90% of rare diseases still lack an FDA-approved drug. The continued dearth of rare disease drugs is a result of a combination of factors including challenges with designing clinical trials in what are exceedingly small patient populations and the difficulty of endpoint selection, particularly when there is limited understanding of the natural history of so many rare diseases. The goal of the ARC program is to address these and other challenges.

ARC Areas of Focus

The ARC program will entail four key areas of focus:

1. Regulatory Science, including around clinical trial design, endpoint selection and interpretation of clinical trial data;
2. Rare disease regulatory policy;
3. Internal coordination, including across medical product centers; and
4. External outreach and coordination across the array of rare disease stakeholders, including academic medical centers, industry and patient organizations.

CDER's ARC Program is governed by leadership across the Office of the Center Director, the Office of New Drugs, and the Office of Translational Sciences, while CDER's Rare Diseases Team will manage the program. The Rare Diseases Team will also double its staff to help handle the added workload. As Dr. Cavazzoni indicated, the staff will "wear multiple hats" working on both the efforts of the ARC Program, as well as the efforts of the review divisions. Importantly, the program allows staff to remain within the current structure of CDER, which maximizes both efficiency and available resources.

Conclusion

The launch of the ARC Program, in the midst of the continuing pandemic, underscores the FDA's longstanding commitment to supporting rare disease drug development. Although the program is founded in CDER, it will certainly help to foster even more coordination of rare disease activities across all medical product centers and the Commissioner's office, not just those

within CDER. Stakeholders across the rare disease community have reason to be optimistic that the ARC Program will lead to significant progress in the years ahead.

Sources: https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/rare-diseases-team: https://pink.pharmaintelligence.informa.com/PS146171/Dont-Call-It-A-Center-Of-Excellence-Accelerating-Rare-Disease-Cures-Program-Launched-By-CDER