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Cystic Fibrosis Research News

Title:

BUILDING GLOBAL DEVELOPMENT STRATEGIES FOR CF THERAPEUTICS DURING A TRANSITIONAL CFTR MODULATOR ERA

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What was your research question?

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We wanted to discuss the impact of modulator therapies (e.g. Kalydeco® and Trikafta/Kaftrio™) on the CF research landscape and identify global research priorities to encourage and support the future development of new CF therapies.

Why is this important?

The CF community is experiencing many changes with the discovery of modulator therapies for which up to 90% of the CF population may be eligible. While these modulator therapies are very promising, we need to encourage global drug development efforts to continue in CF. We have heard from the CF Community that this is important for many reasons: to ensure access to disease altering therapies for all people with CF, to have multiple modulator therapy options for people with CF given varied individual response, and to develop therapies that address the symptoms of CF such as infection and inflammation.

What did you do?

We brought together advisors from multiple countries and clinical trial networks for a one-day workshop at the 2019 North American Cystic Fibrosis Conference. Focusing on the areas of CFTR modulators, anti-infectives, anti-inflammatories, and paediatric drug development, the goal of the workshop was to identify the key drug development questions to promote further development of these therapies for all persons with CF.

What did you find?

Key priorities to support future global drug development efforts were identified at the workshop, in particular the need for more flexibility in future drug development. Developing a new therapy when a similar therapy is already available to people with CF poses one of the more difficult scenarios and one requiring more flexibility. Regular communication between industry, academic investigators, patient organizations, regulatory agencies and CF community representatives will be needed to ensure feasible and informative clinical trials as the landscape of CF care continues to change and in particular with the increased availability of modulator therapies.

What does this mean and reasons for caution?

The collaboration between the global CF research community demonstrated at this workshop and the priorities identified will provide a framework to support and advance the CF drug development pipeline. A limitation to this work, however, was that the workshop was limited to academic researchers only and did not include industry, regulatory agencies or CF community representatives. Therefore, the discussion may not completely represent the

opinion of all groups involved in CF research. Additionally, while consensus was gained in many areas, distinct differences exist between countries and their clinical trial networks which will impact the research priorities for their patient populations such as access to modulator therapies and regulatory requirement differences.

What's next?

The CF global research community will continue their collaboration and will partner with industry and regulators to identify areas for harmonization and efficiencies to support the advancement of a promising drug development pipeline.

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