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Cystic Fibrosis Research News

Title:
A Model for Oversight of Rare Disease Studies: the 25-Year Experience of the Cystic Fibrosis Foundation Data Safety Monitoring Board

Lay Title:
Oversight of Rare Disease Studies: Experience of the CF Foundation DSMB

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What was your research question?
To describe the processes of developing and maintaining the CF Foundation Data Safety Monitoring Board (CFF-DSMB) over the past 25 years.

Why is this important?
A top priority of the CF Foundation Therapeutics Development Network (CFF-TDN) is to protect the safety of patients participating in clinical trials. In 1999, the CF Foundation and the CFF-TDN developed a CF specific DSMB. The CFF-DSMB ensures oversight of clinical trials by professionals familiar with the unique aspects of CF. This paper describes the activities of the CFF-DSMB over the past 25 years during which time we have seen data from approximately 34,000 participant entries and 250 trials. This paper highlights the advantages and challenges of a disease specific DSMB.

What did you do?
This paper outlines the membership of the CFF-DSMB with discussion of the importance of community members. We reviewed how CFF-DSMB members are trained, ongoing education, quality improvement, and the role of having multi-disciplinary members on the DSMB.

What did you find?
Over the past 25 years, the CFF-DSMB has provided safety monitoring for 247 studies with over half of them having international enrollment. In reviewing our experience, we were able

to describe the different ways that the CFF-DSMB has been a part of the clinical trial landscape. The CFF-DSMB has grown from a small group of CF clinician scientists to a well-integrated organization that provides high quality oversight of the majority of CF clinical trials conducted in the US and internationally.

What does this mean and reasons for caution?

The CFF-DSMB's involvement in the CF research has supported a high volume of studies to move rapidly yet safely through the CF drug discovery pipeline. This has contributed to the marked improvement in survival and quality of life for people with CF. The success of the CFF-DSMB can serve as a model for other groups who are dedicated to developing treatments for rare diseases.

What's next?

As the therapeutic landscape moves into the arena of nucleic acid-based studies the CFF-DSMB will continually be evaluating its role in monitoring the safety of early phase studies and the need for long term follow up.

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