

Cystic Fibrosis Research News

Title:

Aligning patients' needs towards a comprehensive CF research program.

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What was your research question?

How was the Dutch research program created and executed. This research program considered the patients' perspective and prioritized the patients' unmet needs in a structured and transparent way.

Why is this important?

It is important to include the patients' perspective, when prioritizing a research program, because scientific work can then add more value to the patients' lives. Once the patients' priorities are understood, clinicians and researchers can be brought into the discussion. Close cooperation between these stakeholders, can lead to a very effective research program.

What did you do?

We described the process that the Dutch CF Foundation followed, to devise a research agenda that was prioritized by the patients. Then these priorities were discussed with researchers. A group of scientists, clinicians and patients created a research program called "HIT CF", which was executed over several years. The CF community was also highly involved in HIT CF, including reviewing and monitoring the program and the projects, and in fundraising. Overall, a translational approach was chosen, in which observations in patient care lead to basic scientific questions for the laboratory, and the answers from the laboratory were subsequently verified in patient care.

What did you find?

This approach urged clinicians and scientists to interact continuously and intensively, leading to the mutual dependency of different study groups. This 'bed to bench to bed' approach



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stimulated profound interactions between CF patients, clinicians and scientists, and had a continuous focus on clinically and socially relevant outcomes for all research efforts within the program. The result was a patient driven research program, that was executed successfully with positive impact for patients. For example, questions from patients concerning personalized medication were addressed in studies with organoids, that resulted in clinical trials and sometimes even reimbursement of off label drugs.

What does this mean and reasons for caution?

An effective research program could be created, if patient organizations are able to organize the process in a structured and professional way, maintaining the patients' perspective as the key factor.

What's next?

It is important to collect more case studies and develop an acknowledged theory with regard to structurally embedding the patients' perspective in research.

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