Cystic Fibrosis Research News

Title:
Fundamental and translational research in Cystic Fibrosis – why we still need it

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Fundamental and translational research in Cystic Fibrosis – why we still need it

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What was your research question?
Here we addressed the most relevant topics covered at the European Cystic Fibrosis Society Basic Science Conference, that was held in Albufeira, Portugal, from March 30th to April 2nd 2022, as they were presented by the participants and summarized in a series of mini-reviews in the journal.

Why is this important?
Although we have witnessed amazing progress in the CF field in the last 10 to 15 years, especially with the development and approval of drugs that help people with CF, the disease is not yet cured. In addition, not all individuals with CF are eligible to take these drugs. Thus, it is critical that we continue to focus on fundamental science, which will help us develop new therapies for everyone suffering with this disease and cure those whose health is improved by existing drugs.

What did you do?
We have provided an overview for the topics that were discussed at the meeting and that were then developed in the eight mini-reviews to be published as a Special Issue of JCF. We discuss what still puzzles researchers on the CFTR protein, which is defective or absent in CF, what the many consequences of CF are, and the development of novel drugs to treat all people with CF.

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What did you find?

We focused on CFTR to understand how it folds into its shape, how it is affected by drugs, and how it regulates many processes, not only in the airways, but in the gut, the pancreas and the kidney. This is relevant as individuals with CF are living longer and problems in other organs are becoming more "visible". We briefly cover what is wrong outside the cell in which CFTR resides, including infections and communication pathways between cells. Finally, we comment on CFTR gene therapy, as well as other proteins that might be "drugged" to help those with CF.

What does this mean and reasons for caution?

There is hope but the road ahead is still long – alternative therapies may be available in the future, but research is still needed. As was the case for the drugs that are now helping many people with CF, fundamental and translational research needs to be supported, so that new and better therapies continue to emerge.

What's next?

The research CF community is committed to pursuing its work to find a "cure" for CF – to continue paving the way, so that every individual with CF will have access to transformative therapies.

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