

This questionnaire is for people with cystic fibrosis (CF) or parents/caregivers of others who have CF and who are taking pancreatic enzyme replacement therapy (PERT). The researcher is Synspira Therapeutics, a company who is committed to CF research and advancing new therapies. Synspira is developing a new pancreatic enzyme that intends to simplify dosing, support long-term outcomes and is non-porcine based. This survey will take about 7 minutes to complete. Your information and opinions are confidential, nothing you say will be attached to your name. To ensure that we can share our learnings, your anonymized responses to these questions may be included and/or aggregated for the purposes of research, education, or program communications.

Background for questionnaire

About 85-90% of people with CF have exocrine pancreatic insufficiency (EPI), meaning their pancreas does not make enough digestive enzymes to break down food so it can be absorbed and used as nutrients and energy. People with EPI use pancreatic enzyme replacement therapy (PERT; commonly called “enzymes”) to help them better digest (break down) fats, proteins, and carbohydrates (parts of food that supply calories and micronutrients) so they can be absorbed to help maintain a healthy weight and overall nutritional status. PERTs need to be taken with all meals and snacks.

A PERT product that aims to simplify dosing, support long-term outcomes and is non-porcine based (not derived from pig pancreas like current PERT products) is being researched. Early phase research studies for a new drug product traditionally involve an intensive 24-hour period where repeated collections of samples (e.g., blood sample(s)), and assessments (e.g., physical exam(s)), are required. Innovations in clinical research studies have made it possible to allow for flexibility in study designs to shorten these clinic visits all while continuing to meet the requirements of the FDA and keeping in mind the concerns of the community.

About the researcher and the aims of this questionnaire

The aims of this survey are:

1. To understand if different settings for research visits are appealing to people with CF.
2. To gain your insights into how best to design a clinical study for a new pancreatic enzyme replacement product (PERTs; commonly called “enzymes”).
3. To better understand the extent to which people with CF would like to participate in research studies for new enzyme.